20 August 2021

THE DOMINO EFFECT

Trikafta was recommended for reimbursement today for people 12+ years with one F508del gene mutation and one minimal function mutation. This is great news for 390+ Australians who have not had a CFTR modulator prior to Trikafta.

However, Trikafta was not recommended for reimbursement for people F508del homozygous and F508del heterozygous with a Residual or Gating mutation. This is a crushing blow, because Australians with CF and their families have been waiting for Trikafta since it became available in the US in October 2019.

The dispute continues over the numbers of eligible patients even though the Australian Cystic Fibrosis Data Registry (ACFDR) has provided ample data on eligibility (indexed by gene mutation) to all parties. CFA has also reached out to our overseas partners in the hope of securing data to speed up decisions and further enhance our argument.

Other roadblocks in the ongoing Trikafta negotiations include onboarding rates, long term data and questions around discontinuation. However, our considered position here at CFA is that both sides are simply jockeying for their preferred price.

CFA would like to see contracts promptly signed so that people with one F508del gene mutation and one minimal function mutation can access a CFTR modulator. We have no assurances that this will happen, and we fear that access may be delayed until a 'Trikafta Deal' is done for the entire F/any CF population.

Minister Hunt said today that he welcomed the PBAC recommendation for Trikafta and that he was heartened that the PBAC had recommended a PBS listing for people with cystic fibrosis with one F508del mutation and a minimal mutation and added " We have written to Vertex to outline our clear intent to list this medicine on the PBS as soon as possible and have called on the global executive of Vertex to work in partnership with the Government and the Australian community".

"We will continue to work towards broadening access to promising new treatments for Australians with CF. Our Government has provided access to Kalydeco, Orkambi and Symdeko and we are committed to fund all new CF treatments once recommended by the PBAC." Minister Hunt added.

It is true that the partial PBAC recommendation is a strong step in the right direction, CFA commends the PBAC for a smart decision.

Sadly CF lives will still be lost, and families destroyed if protracted negotiation continues for the remaining brackets of required Trikafta funding.

The solution is clear:

- The PBAC must recommend Trikafta for reimbursement for the whole CF community 12+
- The ACFDR provides the Dept of Health with quarterly data relating to the number of patients on Trikafta and those who have discontinued treatment
- The Government must reimburse Vertex for all patients taking Trikafta during that period.

This is a good solution for people with CF and for the Australian taxpayer. The usual gatekeepers will tell us that the situation is more complicated, but this is an empty prevarication. The MAP (Managed Access Program) has proven perfectly capable of providing the commercial details around our simple scaffold. Trikafta is feasible in Australia just as it has been elsewhere.

Now is the time for common sense to prevail and level-headed consumers may be needed at the table to get a resolution. CFA is ready and willing to participate and we know that our community will step forward as well.

There are two positive that we can take from today's PBSC ruling

1. Trikafta is recognised by our regulatory body as a highly effective treatment for CF
2. People with one F508del and one minimal function mutation now have a CFTR modulator.

We don't know when Vertex will return to the PBAC, but November is looking like the best case scenario. As soon as CFA knows we will share.

The CF Community has tipped our first domino in the fight for Trikafta, and more are sure to follow as a result. More funding, more age groups, more mutation categories. Progress in this fight has always been made in increments and the Domino Effect is now, officially, in play. Game on.

Kind regards

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