

5 May 2021

Trikafta Update



It is almost a fortnight since we heard the disappointing news that Trikafta had received a 'deferral' from the Pharmaceutical Benefits Advisory Committee (PBAC). Since that time Cystic Fibrosis Australia (CFA) has been meeting with key stakeholders and working behind the scenes to try and get all parties to come together for the benefit of the cystic fibrosis (CF) community.

CFA has called a stakeholder meeting for early June however I think it is important to look at the current most pressing issues ...

1. **The Numbers** - CFA has provided Vertex and the Department of Health with the latest data about our CF population. We have been able to use the Australian Cystic Fibrosis Data Registry (ACFDR) to identify numbers and genotypes. We have also offered the ACFDR resources to all parties to encourage them to overcome this sticking point.
2. **The Long Term Data** - The PBAC is concerned about the uncertain 'safety and effectiveness of Trikafta beyond 48 weeks'. The PBAC's suggestion of 'an appropriate Managed Access Program' would allow the CF community access to Trikafta now at a certain price and then once the long term data (48+ weeks) is presented by Vertex further price negotiations could take place. There are 'risk share arrangements' in place with Orkambi and Symdeko so why should Trikafta be any different.
3. **Comparators** – We expect people on Kalydeco, Orkambi and Symdeko over the age of 12 years will all move to Trikafta. Some people may not tolerate Trikafta as it includes the some of the same drugs as Orkambi and Symdeko. Vertex and the PBAC need to agree what is a fair cost for
 - a. new patients who have not previously had access to a modulator
 - b. those who stopped taking Orkambi and Symdeko but maybe able to tolerate Trikafta plus
 - c. those who are transferring from Kalydeco, Orkambi and Symdeko.

'Cost-effectiveness' needs to be linked to drug effectiveness, safety and tolerance and once again more data is needed.

Vertex confirmed this week that the Australian price for Trikafta was \$277,875. It is important to keep in mind that this is the price before PBAC and Dept of Health negotiations begin.

The CF community is truly global and so if you know anyone who could share their experiences of moving from Kalydeco, Orkambi or Symdeko to Trikafta then we would love to hear about it. CFA will also be reaching out to our colleagues offshore.

I am sorry I cannot report that a 'deal is done' but I truly believe that compassion will prevail. Australians will get Trikafta and CFA will continue to fight to make it happen.

Kind regards

A handwritten signature in black ink, appearing to read 'Nettie Burke'.

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