KEY MESSAGES

1. We're thrilled that Trikafta could be listed on the March 2021 PBAC meeting agenda. This third generation CFTR modulator is a game changer and major milestone in the treatment of CF.
2. Trikafta is designed to treat the underlying cause of CF in people with two copies of the F508del mutation and those with only one copy of F508del. This equates to approx. 90% of the CF population in Australia.
3. Trikafta can prevent permanent, irreversible lung damage.
4. CF patients not on a CFTR modulator can experience a 1-3% average decline in lung function per year. Maintaining or improving lung function is a significant clinical benefit.
5. Even when a CF patient does everything right, they will still experience recurrent pulmonary exacerbations.
6. Trikafta corrects the function of the CFTR protein. Trikafta is more effective than Orkambi and Symdeko.
7. Trikafta significantly improvement in pulmonary exacerbations and BMI and reduces hospitalisation and antibiotic use.
8. Trikafta is highly effective for both adults and children with CF. The TGA and PBAC are currently reviewing Trikafta for Australians 12 years and over.
9. CFA 's Australian Cystic Fibrosis Data Registry gathers information safety and efficacy data (post marketing surveillance) on Orkambi and Symdeko. We are ready to provide the same service for Trikafta.
10. Patient responses to Trikafta may vary and so clinicians are best suited to determine whether the drug is suitable for each patient.
11. The 24-week trial results clearly showed that lung damage was slowed. Reduced lung capacity is a major cause of morbidity and mortality.
12. CFA is concerned with some of the Compassionate Access criteria for Trikafta. We believe that children up to the age of 18 years should be eligible with a lung function of under 50%. Those who are most vulnerable and those who could experience significant long-term preservation of lung function need Compassionate Access immediately.
13. We urge the PBAC to evaluate the totality of evidence of patient response, including but not limited to improvements in FEV_1, reductions in pulmonary exacerbations, increases in BMI, reductions in hospitalizations, and improvements in patient-reported outcomes.
14. Imperfect adherence should not preclude a patient's ability to gain access to this lifesaving therapy indefinitely.
15. We must hold our politician's consciences to the fire, so they don't shy away from the tough decisions.
16. This time around let's not allow people with CF to be caught between commercial negotiation between the Government and Vertex.
17. If negotiations stall CFA has agreed to bring the stakeholders to the table to find an innovative solution to the funding of Trikafta.
18. We live in the lucky country and Australian's are denied access to life saving drugs because of cost.
19. Clinical trials have shown transformative improvements in the lives of people with cystic fibrosis.
20. PBS funding is not capped, and the current government has a policy to list all medicines on the PBS when recommended by the PBAC.