



FACTS

Important messages that the PBAC needs to hear:

1. Australians on ORKAMBI are doing extremely well.
2. Lives have been changed and extended by 23 years.
3. 185 Little Australians need ORKAMBI.
4. ORKAMBI improves lung function and reduce exacerbations, hospitalisation and antibiotic use.
5. ORKAMBI had a positive effect on the mental health of people with CF leading to social inclusion and a reduction in personal and family stress.
6. ORKAMBI for 2-year olds will ensure that their lungs remain as healthy as possible.
7. Young Australians will be well enough to attend school and therefore not suffer from 'late effects' in the education.
8. ORKAMBI makes a difference to the length and quality of people's lives.
9. Every day a person is denied access to ORKAMBI is another day of life limiting - and irreversible - lung damage.
10. ORKAMBI has been approved in the USA, Ireland, Germany, Austria, and Denmark, and young Australians should be granted the same access.
11. ORKAMBI for these younger children will be in the form of oral granules instead of the typical tablets to ease ingestion. The oral granules are similar in size to flour particles and should be mixed with 1 teaspoon (5 mL) of soft food or a liquid.
12. "For the first time, children ages 2 through 5 who have the most common form of CF have a treatment for the underlying cause of their disease," Nettie Burke said today.
13. "It is vital that we treat the underlying cause of CF as early as possible and we need this approval in order to bring effective medicines to all Australians living with CF," Nettie said.
14. The results from a Phase 3 open-label study assessed ORKAMBI's safety in CF patients aged 2 and older with two copies of the *F508del-CFTR* mutation. Participants were medicated every 12 hours, according to their age and weight.
15. The trial showed that ORKAMBI was generally safe and well tolerated for 24 weeks, with children aged 2 to 5 demonstrating a similar safety profile to those who were 6 and older.
16. One secondary endpoint of the study, the levels of sweat chloride, a parameter commonly used to diagnose the disease, improved after 24 weeks

of treatment. Other secondary endpoints, including growth parameters, also improved with the medication.

17. Common adverse events included a cough, affecting 63% of the children in the cohort. Most of the adverse events reported were mild or moderate in severity and similar to those found in patients six years and older.
18. The PBAC has already confirmed the safety and efficacy of ORKAMBI for people with CF aged 6 years and older
19. Clinical trials have proven that lung function is preserved and further decline is halted.
20. 96-week trial outcomes in people aged 12+ clearly showed that lung damage was slowed by an average of 40%.
21. Reduced lung capacity is a major cause of morbidity and mortality.