MEDIA RELEASE

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A Tough Pill to Swallow … ACCESS DEFERRED

Trikafta was not recommended for reimbursement today by the Pharmaceutical Benefits Advisory Committee (PBAC). Having seen the outstanding clinical trial data we can only surmise that once again 'money' is the stumbling block.

"Both sides should know better than to play with the future of living, suffering human beings while they sit back and bicker about money," said Nettie Burke, the CEO of Cystic Fibrosis Australia.

Cystic Fibrosis Australia (CFA) is not suggesting the Government agree to pay the exorbitant list price of $410,000 (est.) per person however we do believe that Vertex is capable of providing Compassionate Access to all eligible Australians while negotiations continue.

ICER* has stated publicly that Trikafta is 'well above commonly-accepted cost-effectiveness thresholds' and at $1120 per day pp we have to agree. ICER estimates that Vertex will earn US$6.6 billion from Trikafta alone by 2023.

"Last night I was on the phone to a mother whose child could barely go to school this year. I had to try and explain to her that the most effective CF drug in the world could turn out to be just too pricey for Australian kids."

"CF lives should not be lost because of craven commercialisation. A few months of Compassionate Access should not be too big a pill to swallow for a company whose share price currently sits at around AUS$278." Nettie added

CFA will be requesting access to Vertex's PBAC Submission and the subsequent PBAC response documents.

"We strongly believe that when lives are at stake then we must hold all stakeholders to account. We will also be holding a stakeholder meeting to get all critical protagonists at the table to share their stumbling blocks and demands," Nettie Burke said.

Until then, CFA's position is that we want immediate Compassionate Access for all eligible Australians while commercial negotiations continue. If you would like to speak to a member of the CF community and hear some 'real life challenges' then call 0404 034 294.

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For further information
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*ICER is a group of independent experts who would review and analyse of the clinical trial evidence, listen to patient testimony, and deliberate over the clinical and economic value of health interventions. https://www.cysticfibrosis.org.au/advocacy/trikafta-advocacy-plan

ABOUT CYSTIC FIBROSIS

Cystic Fibrosis (CF) is a rare, life-shortening genetic disease affecting approximately 75,000 people worldwide and 3,500 people in Australia. CF is a progressive, multi-system disease that affects the lungs, liver, GI tract, sinuses, sweat glands, pancreas and reproductive tract.

CF is caused by a defective and/or missing CFTR protein (cystic fibrosis transmembrane conductance regulator) resulting from certain mutations in the CFTR gene.

Children must inherit two defective CFTR genes — one from each parent — to have CF. While there are more than 2,000 different types of CFTR mutations that can cause the disease, the vast majority of all people with CF have at least one F508del mutation.

These mutations, which can be determined by a genetic test, or genotyping test, lead to CF by creating non-working and/or too few CFTR proteins at the cell surface. The defective function and/or absence of CFTR protein results in poor flow of salt and water into and out of the cells in a number of organs.

This leads to the build up of abnormally thick, sticky mucus in many parts of the body and can cause chronic lung infections and progressive lung damage in many patients that eventually leads to death. The overall predicted (median) survival of people with CF in Australia of 47 years. There is currently no cure.

From birth, a person with CF undergoes constant medical treatments and physiotherapy. People with CF may consume up to 80 capsules daily to help digest food and may need to do up to four hours of airway clearance physiotherapy each day.

In Australia, one in 2,500 babies are born with CF, that is one every four days. On average one in 25 people carry the CF gene and most are unaware that they are carriers. Because carriers of CF are unaffected and therefore show no symptoms, it is hard for them to appreciate that CF may be a real risk.

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