MEDIA RELEASE **26 April 2021**

Patients V's Patience – Never the Twain Shall Meet

The announcement on Friday that the PBAC will not be recommending Trikafta for reimbursement heralds the end of and **patients** with **patience**. We cannot sit idly by while Big Pharma and Government play 'holdout' with people's lives.

We know that every day of delay is a day of lung damage, mental anguish, isolation and progression towards end of life.

Nettie Burke, CEO of Cystic Fibrosis Australia, said today that the solution is clear and it is not new ... ***immediate Compassionate Access for all eligible Australians while commercial negotiations continue.***

"The word **patient** is popularly defined as a person who quietly suffers, someone lying patiently in a bed waiting for interventions from outside experts. The concept of **patience** is an acceptance or tolerance of delays, problems, or suffering without becoming annoyed or anxious".

"The ambiguities of Health Technology Assessment system and the English language are at play here and I choose to believe that the CF community neither should have to lie patiently nor tolerate delays in care," she added.

The vanguard of HIV advocates campaigned on the same platform in the 1980s and in recent years many responsible pharmaceutical companies have generously offered this feature as part of negotiations. Compassionate Access is a norm not a privilege, and lives are on the line here.

"CFA is aware that R&D cost must be met otherwise drug development would cease to exist. However, let's see those costs come out of finalised contracts. Let's see these pharma companies show some heart," Nettie said.

ICER* estimate that Vertex will see Trikafta revenues of US$6.6 by 2023. Compassionate Access to Trikafta will NOT break the bank for Vertex.

Time to call on local power - local pollies and local communities to support our 'Have a Heart' campaign. Simply put the image on your emails, on your socials, on other people socials, on the windows of your car, send CFA photos of your best 'Have a Heart' stance for circulation and use hashtag #haveAheart.

While resubmission and price negotiations continue behind closed doors we need all our stakeholders to know that we are watching, waiting, and hoping they will 'Have a Heart'.

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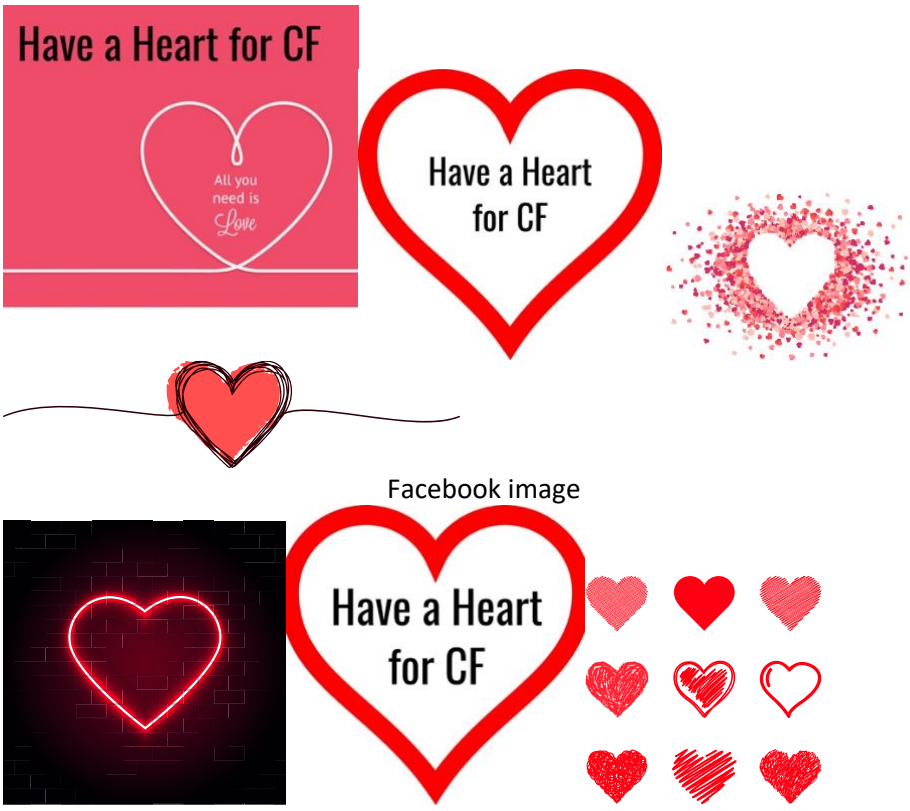
*ICER is a group of independent experts who would review and analyse of the clinical trial evidence, listen to patient testimony, and deliberate over the clinical and economic value of health interventions.
https://www.cysticfibrosis.org.au/advocacy/trikafta-advocacy-plan



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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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