CYSTIC FIBROSIS

CF Fact
cystic fibrosis fact sheet

CFTR Modulator Therapy

Conventional medications for cystic fibrosis (CF) focus on treating the symptoms of CF. Cystic fibrosis transmembrane conductance regulator (CFTR) modulators are a new drug therapy that differ from other CF therapies because they aim to improve or restore the function of the defective CFTR protein made by the CFTR gene.

These therapies are mutation specific and aim to treat the cause of CF and slow the decline in lung function. Different mutations cause different defects in the CFTR protein. The individual modulators act in different ways depending on how the mutant protein affects the cell. It is important to remember that different therapies are suited to different people depending on their mutation and their medical problems. It is necessary to match the right combination of drugs to each individual.

The CFTR protein controls the flow of water and chloride in and out of the cells lining the airways of the lungs and many other organs. Depending on the mutation, the CFTR protein can be present in small amounts or absent completely. The lack of CFTR protein results in sticky mucus in the lungs, damage to the pancreas and problems in other parts of the body.

CFTR Modulators

Kalydeco® (Ivacaftor)
Is used in G551D mutation. Kalydeco® binds to the defective protein, helping to

open the channel so that more chloride and water can move in and out of the cells, thinning the mucus in the lungs and other organs. Available to those eligible over 12 months of age.

Orkambi® (Lumacaftor/Ivacaftor)
A combination corrector therapy that works for people with two copies of the F508 del mutation. Lumacaftor helps the CFTR protein to become the right shape, move to the cell surface and remain there longer. Used with Ivacaftor (Kalydeko®) this allows more chloride and water to flow and reduces the symptoms of CF. This is available to those eligible 6 years of age and over.

Symdeko®
Is a combination of the drugs Tezacaftor® and Lumacaftor (Orkambi®) for people with 2 copies of F508 del who cannot take Orkambi® due to side effects. Tezacaftor® increases the amount of mature CFTR protein delivered to the cell surface.

VX – 445 - is a next generation CFTR corrector designed to restore F508 del CFTR function. Currently phase 3 clinical trials are underway testing the triple combination therapy of VX-445 with Tezacaftor®.

How to Get Involved

Each trial has guidelines about who can participate. They also have inclusion and exclusion criteria which refers to the standards used to decide eligibility.
If you are interested in being involved in a trial, please talk to your CF team.

Useful Resources

- Kalydeco®
- Orkambi®
- Symdeko®
- CFWA Factsheets

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