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Editorial

Cystic Fibrosis: A New Update in the Treatment

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DESCRIPTION

Cystic fibrosis affects one in 17,000 black newborns and one in 31,000 newborns of Asian origin. The rate of cystic fibrosis cases is one in 3,500 newborns among white children in the United States.

Cystic Fibrosis (CF) is the inherited genetic disease which causes thick and stick mucus to build up in organs like pancreas and lungs. Usually in healthy person the mucus is slippery which lines organs and body cavities such as the lungs and the nose whereas in people suffering from cystic fibrosis, it is very difficult to breathe as the thick mucus clogs the airways. In case of children with CF, the children may not be able to absorb enough nutrients from food as the ducts in the pancreas gets blocked and causes problem with digesting the food. Cystic fibrosis is long-lasting and it gets worsen over the time.

Symptoms of Cystic fibrosis include failure to thrive, loose or oily stools, trouble breathing, recurrent wheezing, nagging cough, slow growth, frequent lung infections including bronchitis and pneumonia.

Cystic fibrosis is diagnosed by following methods- like new born screening, sweat test, chest X-rays, genetic tests, sputum culture, lung function tests.

Cystic fibrosis is treated using antibiotics, Mucus thinning medications, Non-Steroidal Anti-Inflammatory Drugs (NSAIDs) like ibuprofen, bronchodilators and CFTR modulators. The surgical procedures include bowel surgery (a section of bowel is removed to relieve blockage in the bowels), feeding tube (where a feeding tube is surgically inserted in the stomach through the nose in order to supply nutrition) or double lung transplant to improve quality of life of patients with Cystic fibrosis.

Recently, a new drug named Trikafta (elexacaftor/ivacaftor/tezacaftor) is approved by USFDA. It is the first triple combination therapy to treat the common cystic fibrosis mutation. This drug is approved for patients who are 12 years and older. In order to use this drug, the patient must have at least one F508del mutation in the CFTR (Cystic Fibrosis Transmembrane conductance Regulator) gene. This gene is estimated to represent 90% of the cystic fibrosis population or roughly 27,000 people in the United States.

This Trikafta usually targets the defective CFTR protein and helps the CFTR gene mutation function made by the protein to work effectively. There are many therapies which are currently available which target the defective protein for treatment but in some patients they have mutations that are ineligible for treatment.

The efficacy of this Trikafta in patients suffering with CF aged 12 years and older was demonstrated in two trials. The first trial includes a 24-week, randomised, double-blind, placebo-controlled trial carried out in 403 patients. These patients had an F508del mutation and also a mutation on the second allele which results in CFTR protein or a CFTR protein which is not responsive to ivacaftor or tezacaftor/ivacaftor alone. The second trial conducted was a four week active controlled trial. The second trial was conducted on 107 patients who had two identical F508del mutations.

The usage of Trikafta induces warnings related to elevated liver function tests when used in combination with other drugs belonging to the category of inducers or inhibitors of liver enzyme called Cytochrome P450 3A4 (CYP3A). There is also the risk of cataracts. Before starting the treatment, it is advisable for the patients and caregivers to have a talk with their health care professionals about these risks.

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