



CFTR MODULATORS IN CROATIA - FIRST EXPERIENCE OF THE ADULT CENTRE FOR CYSTIC FIBROSIS

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Objective:

The CFTR modulator elexacaftor/tezacaftor/ivacaftor (ETI) has been shown to improve lung function, body mass index (BMI) and quality of life in patients with cystic fibrosis (CF). On October 15th, 2021, triple therapy was approved in Croatia for the CF patients (homozygous for F508del and heterozygous F808del/minimal function mutation).

The aim of the study was to evaluate the effectiveness of treatment by triple therapy ETI in adult patients with cystic fibrosis.

Methods:



This retrospective study included adults with CF of the Cystic Fibrosis Centre for children and adults; University Hospital Centre Zagreb. The CF patients were evaluated before treatment and after at least 6 months after initiation of triple therapy. Clinical outcome data comprised lung function, BMI, body composition, adherence to therapy, number of pulmonary exacerbations, use of inhaled antibiotics, chronic colonisation and possible side effects of ETI.

Result:

44 CF patients (25.782 ± 5.24 years old, 54% female, 77% F508del homozygous) were included. We compared spirometry results and BMI before and after at least 6 months of ETI. Significant changes (% change in mean; Wilcoxon p-values reported) were documented for BMI (13%), FEV1% (33%), FVC% (25%), FEF 25-75% (57%) and PEF (34%); all $p < 0.001$. Differences in body composition before and after at least six months of triple therapy were statistically significant (% change in mean; Wilcoxon p-values reported): BMI 13%, fat mass (FM) 11%, fat-free mass (FFM) 5.6%, fat-free mass index (FMI) 4.8, skeletal muscle mass (SMM) 14%, skeletal muscle mass index (SMMI) 13%, total body water (TBW) -5%, Phase angle - 2%; all $p < 0.001$. The use of dornase alfa and inhaled hypertonic saline solution was reduced after CF patients started with triple therapy, but the reduction was not statistically significant ($p = 0.345$, 0.065 , $p < 0.05$). Use of inhaled antibiotics did not statistically change but the frequency of use was reduced. The use of nutritional supplements drinks was reduced, statistically significant ($p < 0.05$). Chronic colonisation with *P.aeruginosa* and *S.aureus* and number of pulmonary exacerbations were reduced; statistically significant ($p < 0.05$). 25% of adult CF patients did not have any side effects. Most frequent was cough (43.18) and headache (18.8%).

Conclusion:

The novel CFTR modulator therapy was associated with a clinically significant improvement in lung function and nutritional status in adult CF patients in Croatia.

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