Hospital Regional Universitario de Málaga

"REAL WORLD" EXPERIENCE OF ELEXACAFTOR/TEZACAFTOR/IVACAFTOR IN THE TREATMENT OF CYSTIC FIBROSIS: EFFECTIVENESS AND SAFETY EVALUATION

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BACKGORUND AND IMPORTANCE

Cystic fibrosis is a life-limiting recessive genetic disorder caused by pathogenic variants in the CFTR gene, resulting in increased viscosity and difficult mucus clearance.

AIM AND OBJECTIVES

Introduction of elexacaftor (ELX) / tezacaftor (TEZ) / ivacaftor (IVA) to clinical practice has brought a change in the clinical approach, since they modulate CFTR. Our aim is to assess effectiveness and security of ELX/TEZ/IVA in our patients.

Data were collected from electronic medical records and pharmacy dispensing programs.

MATERIAL AND METHODS

Observational, retrospective study carried out between March-2020 and September-2022, including all adult patients treated with ELX/TEZ/IVA+IVA in our hospital.

VARIABLES

Sex, age, age of diagnosis, %pFEV1, pulmonary exacerbations, treatment adjustment, adverse events and treatment suspenssion

RESULTS

31 patients (45% male), median age of 31 years (17-45), median age at diagnosis of 4 months (0-38) and median length of ELX/TEZ/IVA + IVA treatment at the momento of the analysis was 9,43 months (4,5-31,4)

%pFEV1	Pulmonary Exacerbations	Treatment Adjustment	Adverse Events	Treatment Suspenssion
%pFEV1 during treatment augmented in 83% patients, slightly decreased in 13% and did not vary in 3,25 patients.	6,5% patients. Required antibiotic treatment but no hospital admission	3,25% patients due to interactions with potent CYP3A4 inhibitors. 3,25% patients due to hepatic insufficiency (Child-Pugh B)	29% patients. Increase of transaminase and/or bilirrubin	3,25% patients temporarily discontinued treatment and 3,2% suspended treatment definitely

Before taking ELE/TEZ/IVA+IVA, 45% patients received TEZ/IVA+IVA as CFTR modulator; 55% did not receive any CFTR modulator.

CONCLUSION AND RELEVANCE

The introduction of ELEX/TEZ/IVA to CF treatment has been a hopeful advance that has shown in our population to have a good safety profile -which can be managed with regular check-ups- and with a good efficacy profile, achieving an increase of %pFVE1in a short time.

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