4CPS-146 'REAL WORLD' EXPERIENCE OFELEXACAFTOR/ TEZACAFTOR/IVACAFTOR IN THE TREATMENT OF CYSTIC FIBROSIS: EFFECTIVENESS AND SAFETY EVALUATION

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Background and Importance Cystic fibrosis (CF) is a life-limiting recessive genetic disorder caused by pathogenic variants in the CFTR (cystic fibrosis transmembrane conductance regulator) gene, resulting in increased viscosity and difficult mucus clearance. Introduction of elexacaftor (ELX)/tezacaftor(TEZ)/ivacaftor(IVA) to clinical practice has brought a change in the clinical approach since they modulate CFTR.

Aim and Objectives To assess effectiveness and security of ELX/TEZ/IVA in patients on a tertiary hospital.

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 included: age, sex, age of diagnose, pulmonary function: measured with % pFEV1 (median percent predicted forced expiratory volume in 1 second) and pulmonary exacerbations; treatment adjustment, adverse events and treatment suspension.

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

Results

Thirty-one patients were included: male 45% (n=14), median of 31 years old (rank 17-45), median age of diagnosis of 4 months (0-38). Before taking ELE/TEZ/IVA+IVA, 45% (n=14) patients received TEZ/IVA+IVA as CFTR modulator; 55% (n=17) did not receive any CFTR modulator. Median length of ELX/TEZ/IVA+IVA treatment at the moment of the analysis was 9.43 months (4.5-31.4).

% pFEV1 during treatment augmented in 83% patients (n=26), slightly decreased in 13% (n=3) and did not vary in 1 patient. Two patients (6.5%) presented pulmonary exacerbations that required antibiotic treatment but not hospital admission.

Two patients (6.5%) required ELX/TEZ/IVA+IVA adjustment: one due to interactions with potent CYP3A4 inhibitors and other because of hepatic insufficiency (Child–Pugh B). Nine (29%) patients presented an increase of transaminase and/or bilirubin in clinical analysis: one patient temporarily discontinued therapy and one suspended treatment definitely.

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.

REFERENCES AND/OR ACKNOWLEDGEMENTS

No conflict of interest.

Conflict of Interest No conflict of interest

4CPS-147 REAL WORLD DATA (RWD) ANALYSIS ON USE OF IMMUNECHECKPOINT INHIBITORS (ICI) FOR NON-SMALL-CELL LUNG CANCER (NSCLC)

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Background and Importance In Italy, monoclonal antibodies acting on programmed cell death protein (PD-1), nivolumab (N) and pembrolizumab (P), or on the PD-L1 ligand, atezolizumab or durvalumab, are authorised for the treatment of NSCLC. Registration RCTs may not give definitive answers regarding the optimal ICI's duration of treatment (DOT). There is evidence that treatment may be interrupted before progression, or before scheduled cycles are completed for different reasons and that potentially affects efficacy. Are the causes for patient discontinuation treatment (TDC) in RCTs and in the real world comparable?

Aim and Objectives Aim: evaluate the appropriateness of treatment choices by analysing DOT with ICI in a cohort of patients with NSCLC

Material and Methods For 27 months data were recorded on patients treated in I-line with P or combinations of P+pemetrexed+platinum chemotherapy (PPC), or in II-line with N. The percentage of PD-L1 expression (PD-L1el) was observed; median DOT was measured, and the data were stratified according to treatment discontinuation causes.

Results A total of 73 patients were treated, 62% men, 38% women, 29% smokers, 3% non-smokers, 40% ex-smokers, and 28% n.a. At the present date 5% of the 73 patients are undergoing treatment and 4% completed all cycles of therapy. Patients were treated with: 33% N, 49% P, and 18% PPC. The PD-L1el in the population treated was for: N 4% >50%, 63% <1%, 33% n.a. versus RTC 77% >5% and 33% >50% (3); for P: <5% 8% and >50% 92% versus RTC 100% >50% (4); for PPC: 67% <5% and 33% < 50% versus RCT-data <=50% 63% and 32% > 50%. Median DOT for P (8 vs 7.9 months), N (5 vs 2.8 months), and PPC (2 vs 9.8 months) in RWD and RCTs respectively. RWD TDC: 96% 7%(4% N, 3% P 17% C), progression 67%(79% N, 53% P and 67% PPC) toxicity 22%(13% N, 28% P and 17% PPC). From RCT data: death/progression (67%N, 47% P, 30.8% PC) and toxicity(3%N, 13.6% P, 13.8%PPC)

Conclusion and Relevance RCT and RWD data are conflicting. Median DOT for P and the N death/progression rate are comparable. The treatment choices made were appropriate, maximising treatment efficacy, while respecting the risk/benefit profile in a population different from that of the RCTs

REFERENCES AND/OR ACKNOWLEDGEMENTS

Conflict of Interest No conflict of interest