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Eligibility of CFTR Modulator [HB1] drugs in patients registered in cystic fibrosis registry of Turkey

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Introduction: CFTR modulatory treatments have resulted in decrease in the frequency of pulmonary exacerbations, increase in quality of life and prolongation of lifespan in CF patients. However, since these drugs are not covered by health insurance in Turkey, eligible patients may sometimes have access to the therapy through lawsuits. In this study, we aimed to determine the eligibility of patients in our country for the modulators.

Method: The data of 35 CF centers registered to CF Registry of Turkey in 2021 were evaluated cross-sectionally. Modulator therapy indications were determined by using Vertexâ algorithm according to the age and CFTR mutations of the patients.

Results: There were a total of 1948 patients registered in 2021, of whom 1930 were alive. Among the surviving patients, 1629 (84.4%) were younger than 18 years and CTFR gene analysis was performed on a total of 1841 (95.4%) patients. Mutations were detected in one allele in 10.7% (198 patients), in both alleles in 79% (1455 patients) of the patients, and no mutations were detected in 188 (10.2%) patients. A total of 855 patients (46.4% of those who underwent gene analysis, 51.7% of patients whose at least 1 mutation was detected) were eligible for the drugs. Considering the indications for more than one drug, the most appropriate drug was found to be elexacaftor/tezacaftor/ivacaftor for 486 patients (26.4%), followed by ivacaftor for 327 patients (17.7%) and lumacaftor/ivacaftor for 42 patients (2%). Among the surviving patients, when one or more drug indications were evaluated, 633 (34.38%) were eligible for elexacaftor/tezacaftor/ivacaftor, 482 (26.18%) for tezacaftor/ivacaftor, 376 (20.42%) for ivacaftor, and 195 (10.59%) for lumacaftor/ivacaftor.

Conclusion: Only almost half of the registered CF patients in our country are eligible for CFTR modulator drugs. There is a need for of new modulator drugs for the rest of our patients who are not eligible for current drugs.

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Interpretation of spirometry parameters in adult patients with cystic fibrosis in North Macedonia

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Objectives: Assessment of lung function, the flow in large, medium and small airways through the analysis of functional diagnostics-spirometry and its parameters.

Methods: We analyzed data from hospital records for 2022year, 25adult CF (cystic fibrosis) patients aged 18–45 were included, 14 men(56%), 11 women (44%). Sporometric parameters were monitored: FVC(vital capacity), FEV1(forced expiratory volume in 1 second), FEF1/FVC (Tiffno), MEF25–75%(forced expiratory flow at 25–75% of the vital capacity). From microbiological analyses-sputum and chronic bacterial isolates were analyzed

Results: In 5 patients (20%) normal pulmonary function FEV1, FVC>80%, Tiffno>70%). Type of ventilatory insufficiency: combined type (FEV1, FVC, Tiffno<70–80%) in 10(40%) patients, obstructive type (FVC>80%, FEV1, Tiffno<70–80%) in 6(24%), restrictive type (FVC, FEV1<80%, Tiffno >70%) in 4(16%) patients. Degree of severity according to FEV1: Mild (FEV1>80%), in 6 patients (24%), moderate(FEV1 = 50–79%) in 9(36%) patients, severe (FEV1 = 30–49%), in 9(36%), very severe (FEV1<30%), in 1 patient (4%). The flow in the small airways (MEF25–75%) is regulated, over 65%, in 3 patients (12%), while it is reduced in 22 patients (88%). From sputum for microbiology, Pseudomonas aeruginosa(PsA) in 11(44%), MRSA (Methicillin-resistant Staphylococcus aureus) in 3(12%), Both bacteria in 2(8%) patients, Staphylococcus aureus(SA) in 2(8%), SA and PsA in 4 pac (16%) and Candida spp in 3 pac (12%).

Conclusion: Spirometry as a functional diagnostic is of particular importance for the assessment of lung function. These patients mostly have a combined type of ventilatory insufficiency of moderate and severe degree, with significant involvement of the small airways and reduced flow in them.