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Cystic Fibrosis Patients Eligible for Modulator Drugs: Data from Cystic Fibrosis Registry of Turkey

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Better understanding of CFTR biology has led to the development of modulator drugs. Lumacaftor/Ivacaftor (L/T) is approved for people ages ≥2 who have two copies of the F508del mutation. Tezacaftor/Ivacaftor (T/I) is approved for people ages ≥12 who have two copies of the F508del mutation and also approved for people who have a single copy of one of 26 specific mutations. Ivacaftor (I) is approved for people ages ≥1 who have a single copy of one of the mutations approved for T/I and additionally for 12 specific mutations. We aimed to find out the number of patients recorded in Cystic Fibrosis Registry of Turkey (CFRT) in 2018 who are eligible for modulator therapy. Of 1170 patients, 128 are homozygote F508del (22 mths-36 yrs), and 123 (10.51%) are aged ≥2 yrs and eligible for L/T. Among 128 patients with homozygote F508del, 48 are aged ≥12 yrs and among 42 patients (1-31 yrs) who have one or two copies of 3849+10kbC→T, A455E, D110H, D579G, F1052V, R74W, R347H, S945L, 13 are aged ≥12 yrs, and totally 61 (5.21%) patients are eligible for T/I. Finally, total 68 (5.81%) patients (1-31 yrs) have one or two copies of D110H, G178R, G1069R, G1349D, R117H and S549R mutations in addition to the mutations stated in T/I group and all of them are older than one year old and eligible for I therapy. According to the data, approximately one fifth of CF patients are eligible for modulator drug therapy. Compared to North European and North American CF patients, less Turkish patients are eligible for modulator therapies, so new treatment modalities are necessary for them.

Session:
Treatments, adherence and psychosocial aspects of cystic fibrosis (Thematic poster)
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