10.14 Not Always What It Seems: A Cross-Sectional Study Looking at Cystic Fibrosis Patients not prescribed Cystic Fibrosis Transmembrane Receptor Modulator (CFTR) Therapy.

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Background: Life-changing CFTR modulator therapy eligibility is determined by genotype, with approximately 10% of patients worldwide predicted to be ineligible (1).

Methods: This cross-sectional study assessed the characteristics of CF patients not prescribed CFTR-modulators attending our adult CF service on June 1st 2022, the reason why, clinical outcomes including: percentage predicted forced expiratory volume in one second, body mass index, number of infective pulmonary exacerbations and hospitalisations and compared them to those on a CFTR-modulator.

Results: 156 patients were analysed. 17(9.3%) were not prescribed a CFTR-modulator; 15(88.2%) due to personal choice, with only two ineligible due to genotype representing 1.3% of the total eligible cohort. Those not prescribed a CFTR-modulator were older ($p=0.0255$), trended towards a higher median ppFEV1 (92%, ($Z=1.562$, $p= 0.1182$)), had a normal median BMI, 24.6(IQR 6.5), and a lower burden of complications with an association between pancreatic insufficiency and modulator therapy (OR 16.63, $P<0.0001$).

Conclusion: Although at first glance the percentage of patients not prescribed a CFTR-modulator was consistent with the 10% predicted ineligibility worldwide, on closer examination this is not the case. Only 1.3% of patients were ineligible due to genotype. There appears to be a self-selecting group choosing to remain off modulators.

Keywords: CFTR Modulator, Patient choice

Disclosures. Nil

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