primary studies - published, non RCT

Clinical Effectiveness of Elexacaftor/Tezacftor/Ivacaftor in People with Cystic Fibrosis.

Code: PM34784492 **Year:** 2021 **Date:** 2021 **Author:** Nichols DP

Study design (if review, criteria of inclusion for studies)

Post-approval study. Prospective, observational study

Participants

487 PwCF age â‰¥12 years with â‰¥1 F508del allele. Average age was 25.1 years.

Interventions

Elexacaftor/tezacaftor/ivacaftor (ETI)

Outcome measures

Assessments occurred before and 1, 3, and 6 months into ETI therapy. Outcomes included change in ppFEV(1), sweat chloride concentration, body mass index, and self-reported respiratory symptoms.

Main results

Average age was 25.1 years. 44.1% entered the study using tezacaftor/ivacaftor or lumacaftor/ivacaftor while 6.7% were using ivacaftor, consistent with F508del homozygosity and G551D allele, respectively. At 6 months into ETI therapy, ppFEV(1) improved 9.76 percentage points (95% CI 8.76, 10.76) from baseline, CFQ-R Respiratory Domain score improved 20.4 points (95% CI 18.3, 22.5), and sweat chloride decreased -41.7 mmol/L (95% CI 43.8, 39.6). BMI also significantly increased. Changes were larger in those naïve to modulators but substantial in all groups, including those treated with ivacaftor at baseline.

Authors' conclusions

ETI by clinical prescription provided large improvements in lung function, respiratory symptoms, and BMI in a diverse population naïve to modulator drug therapy, using existing two-drug combinations, or using ivacaftor alone. Each group also experienced significant reductions in sweat chloride concentration, which correlated with improved ppFEV((1)) in the overall study population.

http://dx.doi.org/10.1164/rccm.202108-1986OC

See also

Am J Respir Crit Care Med. 2021 Nov 16. doi: 10.1164/rccm.202108-1986OC.

Keywords

CFTR Modulators; Genetic Predisposition to Disease; pharmacological_intervention; placebo; VX-770; VX-661; ivacaftor; Aminophenols; tezacaftor; VX-445; elexacaftor; Trikafta; kaftrio;