

primary studies - published, non RCT

Neonatal screening for cystic fibrosis is beneficial even in the context of modern treatment.

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

Real-world study

Participants

117 people with CF aged 12 and above were recruited to the study. The study was conducted in seven sites in Ireland and the UK. 12 years and older homozygous for the F508del mutation (F508del/F508del) or heterozygous for F508del and a minimum function mutation (F508del/MF) were recruited prior to starting ETI and followed up over 12 months.

Interventions

Elexacaftor/Tezacaftor/Ivacaftor (ETI)

Outcome measures

The primary endpoints were lung clearance (LCI(2.5)) and FEV1. Secondary endpoints included spirometry-controlled chest CT scores. LCI(2.5) was measured using nitrogen multiple breath washout (MBW) at baseline, 6 and 12 months. Spirometry was performed as per ERS/ATS criteria. Spirometry controlled Chest CT scans were performed at baseline and 12 months. CT scans were scored using the Perth Rotterdam Annotated Grid Morphometric Analysis (PRAGMA) system. Other outcome measures include weight, height, Cystic Fibrosis Quality of Life, Revised (CFQ-R) questionnaire and sweat chloride.

Main results

Significant improvements were seen in LCI (-2.5, 95%CI -3.0, -2.0) and ppFEV1 (8.9, 95%CI 7.0 - 10.9), ppFVC (6.6, 95%CI 4.9 - 8.3) and ppFEF25-75% (12.4, 95%CI 7.8 - 17.0). Overall PRAGMA-CF scores reflecting airways disease (-3.46, 95%CI -5.23, -1.69). Scores for trapped air, mucus plugging and bronchial wall thickening improved significantly, but bronchiectasis scores did not. Sweat chloride levels decreased in both F508del/F508del (-43.1, 95%CI -47.4, -38.9) and F508del/MF (-42.8, 95%CI -48.5, -37.2) groups. CFQ-R Respiratory Domain (RD) scores improved by 14.2 points (95%CI 11.3, 17.2). At one year, sweat chloride levels were significantly lower in the F508del/F508del group compared to the F508del/MF group (33.93 v. 53.36, p

Authors' conclusions

ETI is associated with substantial improvements in LCI2.5, spirometry and PRAGMA-CF CT scores in people with CF aged 12 years and older. ETI led to improved nutrition and quality of life. People in the F508del/F508del group have significantly lower sweat chloride on ETI treatment compared to the F508del/MF group. Clinical trial registration available at [www. CLINICALTRIALS: gov](http://www.clinicaltrials.gov), ID: NCT04602468.

<http://dx.doi.org/10.1016/j.jpeds.2005.08.002>

See also

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Keywords

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