primary studies - published, non RCT

Long-term impact of Elexacaftor/Tezacaftor/ivacaftor on pulmonary, nutritional and metabolic outcomes in homozygous F508del cystic fibrosis patients: A real-world cohort study.

Code: PM41578635 **Year:** 2026 **Date:** **Author:** Perrotta N

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

Retrospective study

Participants

112 People with CF (PwCF) homozygous for F508del (median age-31â€‰years) treated with ETI from July 2021 to December 2024.

Interventions

Elexacaftor/tezacaftor/ivacaftor (ETI)

Outcome measures

30-month changes in pulmonary, nutritional, metabolic and inflammatory markers. Clinical, spirometric and biochemical data were collected at baseline and at 6, 12, 24 and 30â€‰months.

Main results

ETI produced sustained lung function gains (percent predicted FEVâ‚•â€‰+â€‰15 points at 24â€‰months, pâ€‰

Authors' conclusions

ETI provides durable multisystem benefits, preserving lung function and improving nutritional and metabolic profiles. However, the shift towards overweight/obesity and biochemical signs of hepatic stress suggests evolving cardiometabolic risks. These findings support early ETI initiation and reinforce the need for ongoing monitoring of nutrition, lipid profile and liver function, together with updated CF care strategies to mitigate long-term cardiometabolic complications.

http://dx.doi.org/10.1002/bcp.70460

See also

Br J Clin Pharmacol. 2026 Jan 23. doi: 10.1002/bcp.70460.

Keywords

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