
primary studies - published RCT

A randomised crossover trial of tezacaftor-ivacaftor for gut dysfunction in cystic fibrosis with magnetic resonance imaging (MRI) outcomes: a pilot study.

Code: PM39139270

Year: 2024 **Date:**

Author: Ng C

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

Randomised, double-blind, placebo-controlled, two-period crossover trial

Participants

13 patients with cystic fibrosis

Interventions

Participants were randomly assigned to treatment sequences AB or BA (A:TEZ/IVA, B:placebo, each 28 days), with a 28-day washout period.

Outcome measures

The primary outcome was oro-caecal transit time (OCTT). Secondary outcomes included MRI metrics, symptoms and stool biomarkers.

Main results

We randomised 13 participants. Before the COVID-19 pandemic 8 participants completed the full protocol and 1 dropped out. The remaining 4 participants followed the amended protocol. There were no significant differences between placebo and TEZ/IVA for OCTT (TEZ/IVA >360minutes [225,>360] vs. placebo 330minutes [285,>360], $p=0.8$) or secondary outcomes. There were no adverse events.

Authors' conclusions

Our data contribute to a research gap in the extra-pulmonary effects of CFTR modulators. We found no effect after TEZ/IVA on MRI metrics of gut function, GI symptoms or stool calprotectin. Effects might be detectable with larger studies, longer treatment or more effective CFTR modulators.

<http://dx.doi.org/10.3310/nihropenres.13510.2>

See also

NIHR Open Res. 2024 Mar 19;3:65. doi: 10.3310/nihropenres.13510.2. eCollection 2023.

Keywords

Adult; Aged; CFTR Modulators; Genetic Predisposition to Disease; pharmacological_intervention; placebo; VX-770; VX-661; ivacaftor; Aminophenols; tezacaftor; Symdeko; Symkevi;