*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.

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### 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.

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### 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; Symdeko; Symkevi;