

primary studies - published RCT

Improved residual fat malabsorption and growth in children with cystic fibrosis treated with a novel oral structured lipid supplement: A randomized controlled trial.

Code: PM32384122

Year: 2020 Date:

Author: Stallings VA

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/10.1371/journal.pone.0232685>

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;