

primary studies - published RCT

Bolus weekly vitamin D3 supplementation impacts gut and airway microbiota in adults with cystic fibrosis: a double-blind, randomized, placebo-controlled clinical trial.

Code: PM29161417

Year: 2017 **Date:**

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

Randomized controlled trial

Participants

6 through 11 years of age with cystic fibrosis (CF) homozygous for the F508del-CFTR mutation or heterozygous with a residual function-CFTR mutation (F/F or F/RF respectively).

Interventions

Participants were randomized 4:1 to tezacaftor/ivacaftor or a blinding group (placebo for F/F, ivacaftor for F/RF).

Outcome measures

The primary endpoint was within-group change from baseline in the lung clearance index 2-5 (LCI(2-5)) through Week 8. Secondary endpoints were change from baseline in sweat chloride (SwCl), cystic fibrosis questionnaire-revised (CFQ-R) respiratory domain score, and safety.

Main results

Sixty-seven participants received at least one study drug dose. Of those, 54 received tezacaftor/ivacaftor (F/F, 42; F/RF, 12), 10 placebo, and 3 ivacaftor; 66 completed the study. The within-group change in LCI(2-5) was significantly reduced (improved) by -0.51 (95% CI: -0.74 , -0.29). SwCl concentration decreased (improved) by -12.3 mmol/L and CFQ-R respiratory domain score increased (improved, nonsignificantly) by 2.3 points. There were no serious adverse events (AEs) or AEs leading to tezacaftor/ivacaftor discontinuation or interruption. The most common AEs ($\geq 10\%$) in participants receiving tezacaftor/ivacaftor were cough, headache, and productive cough.

Authors' conclusions

Tezacaftor/ivacaftor improved lung function (assessed using LCI) and CFTR function (measured by SwCl concentration) in participants 6 through 11 years of age with F/F or F/RF genotypes. Tezacaftor/ivacaftor was safe and well tolerated; no new safety concerns were identified.

<http://dx.doi.org/10.1210/jc.2017-01983>

See also

J Clin Endocrinol Metab. 2017 Nov 16. doi: 10.1210/jc.2017-01983.

Keywords

Children; CFTR Modulators; Genetic Predisposition to Disease; pharmacological_intervention; placebo; VX-770; VX-661; ivacaftor; Aminophenols; tezacaftor; VX-445; elexacaftor; non pharmacological intervention - diagn; Trikafta; kaftrio;