*primary studies - published, non RCT*

# Impact of lumacaftor/ivacaftor and tezacaftor/ivacaftor on treatment response in pulmonary exacerbations of F508del/F508del cystic fibrosis.

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

Secondary analysis of STOP2, a large multicenter randomized controlled trial of antimicrobial treatment durations for adult PWCF presenting with PEx. Propensity score matching was used to compare outcomes

### Participants

People with cystic fibrosis (PWCF). Among 982 PEx events in randomized PWCF, 480 were homozygous for F508del, of whom 289 were receiving lumacaftor/ivacaftor or tezacaftor/ivacaftor at initiation of antibiotic therapy.

### Interventions

Lumacaftor/ivacaftor or tezacaftor/ivacaftor and controls not receiving CFTR modulator therapy.

### Outcome measures

The primary outcome measure was the change in percent predicted FEV(1) (ppFEV(1)) following completion of intravenous (IV) antibiotics, with post-antibiotic changes in symptoms, serum C-reactive protein (CRP) concentrations and weight included as secondary endpoints.

### Main results

Modulator-treated F508del/F508del PWCF did not demonstrate greater improvements in ppFEV(1), symptoms, serum CRP or weight following antibiotic treatment compared to modulator-naïve controls matched for age, sex, baseline ppFEV(1), genotype, body mass index, initial CRP, initial symptoms, exacerbation history, diabetic status, randomization arm and concomitant medical therapy.

### Authors' conclusions

In the acute setting, CFTR modulator therapy with lumacaftor/ivacaftor or tezacaftor/ivacaftor does not convey additional clinical or biochemical advantage above standardized PEx treatment in F508del/F508del PWCF.

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### See also

J Cyst Fibros. 2023 Jul 3:S1569-1993(23)00826-3. doi: 10.1016/j.jcf.2023.06.012.

### Keywords

Aminophenols; Anti-Bacterial Agents; CFTR Modulators; Genetic Predisposition to Disease; pharmacological_intervention; Quinolones; GLPG2737; ivacaftor+lumacaftor; Orkambi; tezacaftor; Symdeko; Symkevi;