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

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Keywords

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