primary studies - published RCT

Empire-CF study: A phase 2 clinical trial of leukotriene A4 hydrolase inhibitor acebilustat in adult subjects with cystic fibrosis.

Code: PM34538755 **Year:** 2021 **Date:** 2021 **Author:** Elborn JS

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

Randomized placebo-controlled trial

Participants

CF patients, 199 subjects. Subjects were stratified by use of concomitant CF transmembrane conductance regulator (CFTR) modulators, baseline percent predicted forced expiratory volume in 1 second (ppFEV(1)) 50-75 and >75, and number of pulmonary exacerbations in the past year (1 or >1)

Interventions

Leukotriene B4 (LTB(4)) is a neutrophil chemoattractant. Subjects were randomized (1:1:1) to once-daily acebilustat 50 mg, 100 mg or placebo for 48 weeks, concomitantly with their current therapeutic regimen.

Outcome measures

Primary endpoints were the change from baseline in ppFEV(1) and safety. Secondary endpoints included the rate of pulmonary exacerbations.

Main results

Overall, 199 subjects were randomized and dosed (acebilustat 50 mg, n=67; acebilustat 100 mg, n=66; placebo, n=66). Baseline demographics and disease profile were well balanced among treatment groups. Acebilustat had no statistically significant effect on the primary endpoint of change in ppFEV(1) at week 48 or the secondary endpoint pulmonary exacerbations. There was a trend towards reduced pulmonary exacerbations in subjects receiving acebilustat in pre-specified populations with ppFEV(1)>75 (35% rate reduction) and those on concomitant CFTR modulator therapy (20% rate reduction). Acebilustat was well tolerated.

Authors' conclusions

Acebilustat did not improve lung function. A trend towards reduced pulmonary exacerbations in subjects with an earlier stage of lung disease suggests a potential effect in this population.

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See also

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Keywords

Acebilustat; Leukotriene antagonists; Anti-Inflammatory Agents - excl Steroids; pharmacological_intervention;