

primary studies - published RCT

# How Many Maneuvers Should We Do for Maximal Inspiratory and Expiratory Muscle Pressure Testing in Children: A Retrospective Review in Children with Cystic Fibrosis.

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

Randomized, double-blind, placebo-controlled Phase 2b trial.

## Participants

PwCF were  $\geq 12$  years old with 2-3 pulmonary exacerbations (PE<sub>x</sub>) treated with intravenous (IV) antibiotics (or 1 PE<sub>x</sub> treated with IV and  $\geq 1$  PE<sub>x</sub> treated with oral antibiotics) in the past year. 447 subjects from 21 countries, mean age was 26.9 (10.3 SD) years, 53.6% were female, 45.2% homozygous for F508del, and 24.9% received CFTR modulators.

## Interventions

Subjects were randomized 2:1:2 to lenabasum 20 mg BID, lenabasum 5 mg BID, or placebo BID.

## Outcome measures

Primary endpoint was rate of Pex

## Main results

PE<sub>x</sub> incidence over 28 weeks was 0.84 for placebo, 0.75 for lenabasum 5 mg BID, and 0.91 for lenabasum 20 mg BID; rates were not lower relative to placebo in the 5 mg (incidence rate ratio (IRR)=0.89, 95% CI 0.66 to 1.19,  $p = 0.44$ ) or the 20 mg group (IRR 1.08, 95% CI 0.86 to 1.37,  $p = 0.51$ ). PE<sub>x</sub> occurred less frequently in participants from Eastern Europe, but there was no evidence of regional variation in treatment efficacy. Lenabasum was well tolerated, without safety signals.

## Authors' conclusions

Lenabasum did not improve key clinical outcomes in this Phase 2b study in pwCF.

<http://dx.doi.org/10.1007/s00408-021-00422-0>

## See also

Lung. 2021 Feb 15. doi: 10.1007/s00408-021-00422-0.

## Keywords

Lenabasum; Cannabinoid receptor agonist; Anti-Inflammatory Agents - excl Steroids; pharmacological\_intervention; Bacterial Infections; Respiratory Tract Infections; Respiratory Tract Diseases; Infection; Exacerbation;