primary studies - published RCT

Lumacaftor/Ivacaftor reduces pulmonary exacerbations in patients irrespective of initial changes in FEV1.

Code: PM30146268 **Year:** 2019 **Date:** 2019 **Author:** McColley SA

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

Post hoc analyses of pooled phase 3 data (NCT01807923, NCT01807949)

Participants

Patients with cystic fibrosis homozygous for F508del.

Interventions

lumacaftor/ivacaftor (LUM/IVA)

Outcome measures

Pulmonary exacerbations (PEx)

Main results

LUM (400mg q12h)/IVA (250mg q12h)-treated patients (n=369) experienced significantly fewer PEx vs placebo, regardless of threshold category. With LUM/IVA, PEx rate per patient per year was 0.60 for those with absolute change in ppFEV1>0 and 0.85 for those with absolute change

Authors' conclusions

LUM/IVA significantly reduced PEx, even in patients without early lung function improvement.

http://dx.doi.org/10.1016/j.jcf.2018.07.011

See also

J Cyst Fibros. 2019 Jan;18(1):94-101. doi: 10.1016/j.jcf.2018.07.011. Epub 2018 Aug 23.

Keywords

Child; Adult; Adolescent; Aminophenols; CFTR Modulators; Genetic Predisposition to Disease; Orkambi; pharmacological_intervention; VX-770; ivacaftor; lumacaftor; VX-809;