primary studies - published, non RCT

Use of lung transplantation survival models to refine patient selection in cystic fibrosis.

Code: PM15695493 **Year:** 2005 **Date:** **Author:** Liou TG

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

Single-center, retrospective study

Participants

The study included 174 CF patients on elexacaftor/tezacaftor/ivacaftor (ETI), six years and older with at least one copy of F508del

Interventions

De-escalating cystic fibrosis

Outcome measures

The primary objective was to assess non-inferiority of supportive therapies de-escalation by comparing the absolute change in percent predicted (ppFEV(1)) from baseline to month 1 versus the absolute change from baseline to month 12 after initiating ETI with patients serving as their own control.

Main results

The study included 174 patients. The mean ppFEV(1) at baseline, month 1, and month 12 was 67%, 78%, and 87% respectively. The mean difference in absolute change in ppFEV(1) from baseline to month 1 compared to baseline to month 12 after the initiation of ETI was 1.53% (95% CI: -0.49 to 3.55)

Authors' conclusions

De-escalating supportive therapies for those on ETI was non-inferior to remaining on all supportive therapies. This suggests that medications may be able to be discontinued under the context of a de-escalation algorithm, which may decrease medication burden and cost and increase quality of life.

http://dx.doi.org/10.1164/rccm.200407-900OC

See also

Am J Respir Crit Care Med. 2005 May 1;171(9):1053-9. Epub 2005 Feb 1.

Keywords

CFTR Modulators; Genetic Predisposition to Disease; pharmacological_intervention; placebo; VX-770; VX-661; ivacaftor; Aminophenols; tezacaftor; VX-445; elexacaftor; Trikafta; kaftrio;