primary studies - published RCT

Sweat chloride as a biomarker of CFTR activity: Proof of concept and ivacaftor clinical trial data.

Code: PM24660233 **Year:** 2014 **Date:** 2014 **Author:** Accurso FJ

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

placebo-controlled, multicenter trial - Authors examined data from a Phase 2 trial (NCT00457821)

Participants

of ivacaftor, a CFTR potentiator, in cystic fibrosis (CF) patients with a G551D mutation

Interventions

Ivacaftor. Sweat chloride and nasal potential difference (NPD) to estimate CFTR activity. Sweat chloride and NPD were secondary endpoints in this placebo-controlled, multicenter trial.

Outcome measures

Differences between dose-groups and assess ivacaftor treatment effects (CFTR activity)

Main results

Within-patient sweat chloride determinations showed sufficient precision to detect differences between dose-groups and assess ivacaftor treatment effects. Analysis of changes in sweat chloride and NPD demonstrated that patients treated with ivacaftor achieved CFTR activity equivalent to approximately 35%-40% of normal.

Authors' conclusions

Sweat chloride is useful in multicenter trials as a biomarker of CFTR activity and to test the effect of CFTR potentiators.

http://onlinelibrary.wiley.com/o/cochrane/clcentral/articles/403/CN-00978403/frame.html

See also

J Cyst Fibros. 2014 Mar;13(2):139-47.

Keywords

Child; Adult; Adolescent; Aminophenols; CFTR Modulators; Genetic Predisposition to Disease; pharmacological_intervention; VX-770; ivacaftor; G551D-CFTR;