primary studies - published RCT

Optimizing nasal potential difference analysis for CFTR modulator development: assessment of ivacaftor in CF subjects with the G551D-CFTR mutation.

Code: PM23922647 **Year:** 2013 **Date:** 2013 **Author:** Rowe SM

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

RCT

Participants

Thirty-nine subjects with CF who also had the G551D-CFTR

Interventions

Patients were randomized to receive ivacaftor (Kalydeco; also known as VX-770) in four doses or placebo twice daily for at least 14 days. All data were analyzed by a single investigator who was blinded to

Outcome measures

All data were analyzed by a single investigator who was blinded to treatment assignment. Three analysis methods were compared to determine the best approach to quantify changes in chloride and sodium transport: (1) the average of both nostrils; (2) the most-polarized nostril at each visit; and (3) the most-polarized nostril at screening carried forward. Parameters of ion transport included the PD change with zero chloride plus isoproterenol (CFTR activity), the basal PD, Ringer's PD, and change in PD with amiloride (measurements of ENaC activity), and the delta NPD (measuring CFTR and ENaC activity).

Main results

The average and most-polarized nostril at each visit were most sensitive to changes in chloride and sodium transport, whereas the most-polarized nostril at screening carried forward was less discriminatory. NPD studies should assess both nostrils rather than a single nostril. Changes in CFTR activity were more readily detected than changes in ENaC activity, and that rigorous standardization was associated with relatively good within-subject reproducibility in placebo-treated subjects (+/- 2.8 mV).

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See also

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Keywords

diagnostic procedures; non pharmacological intervention - diagn; Aminophenols; CFTR Modulators; Genetic Predisposition to Disease; pharmacological_intervention; VX-770; ivacaftor; G551D-CFTR;