*primary studies - published RCT*

# In vivo and in vitro ivacaftor response in cystic fibrosis patients with residual CFTR function: N-of-1 studies.

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

Series of randomized, crossover N-of-1 trials of ivacaftor and placebo

### Participants

CF patients >/=8 years old with potential residual CFTR function (intermediate sweat chloride concentration, pancreatic sufficient, or mild bronchiectasis on chest CT).

### Interventions

Ivacaftor vs placebo

### Outcome measures

Human nasal epithelium (HNE) was obtained via nasal brushing and cultured. Sweat chloride concentration change was the in vivo outcome. Chloride current change in HNE cultures with ivacaftor was the in vitro outcome.

### Main results

Three subjects had decreased sweat chloride concentration (-14.8 to -40.8 mmol/L, P

### Authors' conclusions

Some CF patients with residual CFTR function have decreased sweat chloride concentration with ivacaftor. Increased chloride current in HNE cultures among subjects with decreased sweat chloride concentrations may predict clinical response to ivacaftor. Ivacaftor can increase sweat chloride concentration in certain mutations with unclear clinical effect.

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### See also

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### Keywords

Aminophenols; CFTR Modulators; Genetic Predisposition to Disease; pharmacological_intervention; VX-770; ivacaftor;