

primary studies - published RCT

## Higher tobramycin concentration and vibrating mesh technology can shorten antibiotic treatment time in cystic fibrosis.

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### 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://dx.doi.org/10.1002/ppul.21376>

### See also

Pediatr Pulmonol. 2011 Apr;46(4):401-8. doi: 10.1002/ppul.21376. Epub 2010 Dec 30.

### Keywords

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