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*primary studies - published RCT*

## **A randomized placebo-controlled trial of miglustat in cystic fibrosis based on nasal potential difference.**

**Code:** PM22281182

**Year:** 2012 **Date:** 2012

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

Single-center, randomized, double-blind, placebo-controlled, crossover Phase II study

### **Participants**

11 patients (mean±SD age, 26.3±7.7years) homozygous for the F508del mutation

### **Interventions**

oral miglustat 200mg t.i.d. or placebo for two 8-day cycles separated by a 14-day washout period.

### **Outcome measures**

The primary endpoint was the change in total chloride secretion (TCS) assessed by nasal potential difference.

### **Main results**

No statistically significant changes in TCS, sweat chloride values or FEV(1) were detected. Pharmacokinetic and safety were similar to those observed in patients with other diseases exposed to miglustat.

### **Authors' conclusions**

There was no evidence of a treatment effect on any nasal potential difference variable. Further studies with miglustat need to adequately address criteria for assessment of nasal potential difference.

<http://dx.doi.org/10.1016/j.jcf.2011.12.004>

### **See also**

J Cyst Fibros. 2012 May;11(3):231-6. doi: 10.1016/j.jcf.2011.12.004. Epub 2012 Jan 27.

### **Keywords**

Miglustat; Anti-Inflammatory Agents; pharmacological\_intervention; Anti-Inflammatory Agents - excl Steroids;