primary studies - published RCT

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

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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 200mgt.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;