

primary studies - published RCT

Dornase alpha and exhaled NO in cystic fibrosis.

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

randomized, double-blind, multi-center, 28-day, phase 2 clinical trial

Participants

89 patients with screening FEV(1) \geq 75% of predicted normal value and not treated with inhaled antibiotics for the past 30 days

Interventions

Patients were randomized to receive one of three doses of denufosal (20, 40, or 60 mg) or placebo (normal saline) administered three times daily.

Outcome measures

safety and efficacy. FEV1, FVC, FEF25-75, adverse events

Main results

89 patients were randomized and received the study drug, 94% completed the study, and 98% were compliant with dosing. All treatments were generally well tolerated, with no dose-response trends observed with respect to safety parameters. The most common adverse event was cough (52% of placebo patients and 47% of denufosal patients). Five patients discontinued early due to adverse events, two on placebo and three on denufosal. Denufosal patients (pooling active doses) had significantly higher changes from baseline in FEV(1) ($P = 0.006$), FEF(25%-75%) ($P = 0.008$), FVC ($P = 0.022$), and FEV(1)/FVC ($P = 0.047$) than placebo patients at the end of the study.

Authors' conclusions

Denufosal administered three times daily for 28 days appeared to be safe and well tolerated in this population with mild cystic fibrosis and provided preliminary evidence of potential benefit in lung function.

<http://dx.doi.org/10.1002/ppul.20088>

See also

Pediatr Pulmonol. 2004 Nov;38(5):379-85.

Keywords

Adolescent; Adult; Child; Other drugs; denufosal; Inhalation OR nebulised; nebuliser; non pharmacological intervention - devices OR physiotherapy; pharmacological_intervention;