primary studies - published RCT

A two-year randomized, placebo-controlled trial of dornase alfa in young patients with cystic fibrosis with mild lung function abnormalities.

Code: PM11743506 **Year:** 2001 **Date:** 2001 **Author:** Quan JM

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

Randomised, double-blind parallel placebo controlled trial over 96 weeks

Participants

60 participants withdrew from the study, 472 (out of 474) had follow-up data. The ITT population was 470. Children ages 6 - 10 years (mean age 8.4 years) with FVC > 85% predicted. 239 participants randomized to dornase alfa and 235 to placebo. 410 completed the study. 49 CF centres.

Interventions

Participants treated with 2.5 mg dornase alfa od or placebo.

Outcome measures

Pulmonary function (FEV1, FVC) and exacerbations, deaths, adverse events, change in weight for age. Measurements taken at week 4, 12 and every 12 weeks thereafter.

Main results

Patients were randomized, 239 to dornase alfa and 235 to placebo. At baseline the mean age was 8.4 years, the mean forced expiratory volume in 1 second 95% predicted, the mean forced expiratory flow, midexpiratory phase 85% predicted, and the mean forced vital capacity 102% predicted. At 96 weeks the treatment benefit for dornase alfa compared with placebo in percent predicted (mean +/- SE) was 3.2 +/- 1.2 for forced expiratory volume in 1 second (P =.006), 7.9 +/- 2.3 for forced expiratory flow between 25% and 75% of vital capacity (P =.0008), and 0.7 +/- 1.0 for forced vital capacity (P =.51). The risk of respiratory tract exacerbation was reduced by 34% in patients who received dornase alfa (relative risk 0.66, P =.048). There was no statistically significant difference between the groups in changes in weight-for-age percentile. Adverse event profiles for the treatment groups were similar.

Authors' conclusions

Treatment of young patients with CF with dornase alfa maintains lung function and reduces the risk of exacerbations over a 96-week period.

http://dx.doi.org/10.1067/mpd.2001.118570

See also

J Pediatr. 2001 Dec;139(6):813-20.

Keywords

Child; Deoxyribonuclease; Airway clearance drugs -expectorants- mucolytic- mucociliary-; pharmacological_intervention; placebo; Recombinant Proteins; Respiratory System Agents; Respiratory Tract Diseases; Dornase alpha; Pulmozyme; Inhalation OR nebulised; nebuliser;