primary studies - published RCT

Ursodeoxycholic acid for liver disease associated with cystic fibrosis: a double-blind multicenter trial. The Italian Group for the Study of Ursodeoxycholic Acid in Cystic Fibrosis.

Code: PM8675168 **Year:** 1996 **Date:** 2001 **Author:** Colombo C

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

Open cross-over randomised controlled trial, three 12-week treatment periods, with a 2-week wash out period between each period

Participants

48 children (age range 7.3 to 17 years) randomised, 45 completed first treatment period, 44 completed the second treatment period and 40 completed the third treatment period. The study population comprised children with cystic fibrosis. Inclusion criteria were: age between 5 and 18 years; ability to carry out spirometry; and either current treatment with rhDNase or a forced expiratory volume in 1 second (FEV1) of less than 70% of predicted. Exclusion criteria were: inability to take the trial medication; known hypersensitivity to rhDNase of HS; isolation of Burkholderia cepacia in the sputum; pregnancy; and breastfeeding. Patients had to have no lower-respiratory-tract infection during the 14 days before the start of the study.

Interventions

2.5 mg dornase alfa od, alternate day 2.5 mg dornase alfa and 5 mL 7% hypertonic saline bd.

Outcome measures

Primary outcome was FEV1; secondary outcomes were FVC, number of pulmonary exacerbations, weight gain, quality of life, exercise tolerance and the total costs of hospital and community care.

Main results

Effectiveness results: FEV1, the mean percentage change was 16% (SD = 25) for daily rhDNase, 14% (SD = 22) for alternate-day rhDNase, and 3% (SD = 21) for HS.There was an 8% advantage of daily rhDNase over HS (95% CI: 2 - 14; p=0.01) but the 2% advantage of daily rhDNase over alternate-day rhDNase was not statistically significant (95% CI: -4 - 9; p=0.55). Adjustments for age of the children, treatment period, and quarterly season of the year confirmed the results. No statistically significant differences among the treatments with respect to secondary outcomes. Cost results: in the comparison of daily rhDNase and HS, intervention costs were 1,755 versus 37, other drug costs amounted to 2,271 versus 2,364, and hospital and community costs were 1,668 versus 1,883, respectively. Total costs were 5,694 for daily rhDNase and 4,285 for HS.In the comparison of daily rhDNase and alternate-day rhDNase, intervention costs were 1,749 versus 857, other drug costs amounted to 2,367 versus 2,349, and hospital and community costs were 1,595 versus 1,992, respectively.Total costs were equal to 5,711 for daily rhDNase and 5,198 for HS but the difference (513) was not statistically significant.

Authors' conclusions

Hypertonic saline, delivered by jet nebuliser, is not as effective as daily rhDNase, although there is variation in individual response. There is no evidence of a difference between daily and alternate-day rhDNase.

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See also

Hepatology. 1996 Jun;23(6):1484-90.

Keywords

Adolescent; Child; Deoxyribonuclease; Drug Administration Schedule; Airway clearance drugs -expectorants- mucolytic- mucociliary-; hydration; Hypertonic Solutions; pharmacological_intervention; Recombinant Proteins; Respiratory System Agents; Dornase alpha;

Pulmozyme; Inhalation OR nebulised; nebuliser;