Title A Comparative Study of Hypertonic Saline, Daily and Alternate-day rhDNase in Children with Cystic Fibrosis

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Aim

The objective of this study is to compare the efficacy and cost effectiveness of daily recombinant human deoxyribonuclease (rhDNase), alternate-day rhDNase and hypertonic saline in the treatment of children with cystic fibrosis.

Conclusions and results

Forty-eight children were recruited to the study. Following 12 weeks of treatment, there was a mean increase in FEV1 over baseline of 16%, 14%, and 3% with daily rhDNase, alternate-day rhDNase, and hypertonic saline, respectively. Comparing daily rhDNase with alternate-day rhDNase, there was no evidence of difference between the treatments. However, daily rhDNase showed a significantly greater increase in FEV1 compared with hypertonic saline. The mean difference in total cost between daily rhDNase and alternate-day rhDNase was £513 over the 12-week treatment period, and between daily rhDNase and hypertonic saline it was £1409. None of the other secondary outcome measures showed significant differences between the treatments.

Recommendations

Alternate-day rhDNase appears to be as effective as daily rhDNase in cystic fibrosis and, on average, reduces health service costs. It appears that 7% hypertonic saline is not as effective as daily rhDNase, although there was some variation in individual response.

Methods

This was an open-label, active-treatment, randomized crossover trial. Each patient was allocated consecutively to 12 weeks of treatment with once-daily 2.5 mg rhDNase, alternate-day 2.5 mg rhDNase, or twice-daily 5 ml of 7% hypertonic saline, in random order. There was a 2-week washout period between treatments.

Further research/reviews required

To support the results, a followup long-term parallel trial comparing daily rhDNase with alternate-day rhDNase, which includes a health economic analysis, should be performed.

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