

primary studies - published RCT

A placebo-controlled study of liposome-mediated gene transfer to the nasal epithelium of patients with cystic fibrosis.

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

Randomised controlled trial; parallel design.

Participants

Inclusion criteria: CF; no organ transplantation; 70% of all eligible participants in a longitudinal BMD study recruited after one year of follow-up; no prior treatment with bone sparing agents; BMD Z-score of

Interventions

All participants with pancreatic insufficiency (relevant to all except one in control group) continued long term oral vitamin D (900 IU/day); all participants in both groups received oral calcium (1g daily). Intravenous pamidronate 30 mg every 3 months for 6 months (2 doses).

Outcome measures

BMD (distal radius, ultradistal radius; 0, 6 months; SXA); Adverse events (bone pain); Withdrawals (total, due to adverse events); BMD (lumbar spine; proximal femur (total hip); 0, 6 months; DXA Hologic QDR 4500 Waltham MA); Survival.

Main results

Trial duration planned for 1 year, but was shortened to 6 months because of adverse events. After 6 months of treatment the pamidronate group (n=13) showed a significant increase in absolute BMD compared with the control group (n=15) in the lumbar spine (mean difference 5.8% (CI 2.7% to 8.9%)) and total hip (mean difference 3.0% (CI 0.3% to 5.6%)). However, the pamidronate group showed a reduction in BMD compared with the control group in the distal forearm (mean difference -1.7% (CI -3.7% to 0.3%)). The use of pamidronate was associated with a high incidence of bone pain in non-corticosteroid treated individuals.

Authors' conclusions

Intravenous pamidronate increases axial BMD in adults with cystic fibrosis, but the high incidence of bone pain associated with this treatment might limit its use.

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See also

Gene therapy YR: 1997 VL: 4 NO: 3

Keywords

Adolescent; Adult; Bisphosphonates; Bone Density Conservation Agents; Bone Diseases; Intravenous; pamidronate; pharmacological_intervention; Oral;