primary studies - published RCT

Repeated aerosolized AAV-CFTR for treatment of cystic fibrosis: a randomized placebo-controlled phase 2B trial.

Code: PM17685853 **Year:** 2007 **Date:** 2011 **Author:** Moss RB

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

Preliminary, prospective, single-blind, randomised controlled trial

Participants

Specialist cystic fibrosis centre. Adults recruited from a cystic fibrosis outpatient clinic

Interventions

weekly musculoskeletal treatment for 6 weeks in addition to normal optimal physiotherapy care VS normal optimal physiotherapy care

Outcome measures

Recorded at baseline, 3, 6 and 12 weeks. The outcome measures were posture (thoracic index), chest wall excursion, forced expiratory volume in 1 second (FEV), visual analogue scale for pain, modified shuttle test and Cystic Fibrosis Quality of Life Questionnaire--Section One (physical functioning).

Main results

From a total of 20 subjects, 10 were randomised to each group. Fifty percent of subjects were male, with a median age of 27 years (IQR 25 to 34), median FEV(1) of 1.75 l (IQR 1.4 to 2.4) and median body mass index of 20.8 (IQR 20.0 to 23.5). Baseline differences between groups in thoracic index and modified shuttle test made any differences difficult to interpret, but the results for thoracic index and chest wall excursion at the third rib in the treatment group showed a trend towards improvement. The usefulness of FEV, the visual analogue scale for pain and the Cystic Fibrosis Quality of Life Questionnaire as measures is unclear.

Authors' conclusions

Further musculoskeletal studies in people with cystic fibrosis should consider using thoracic index and a measure of lung function in addition to FEV. The musculoskeletal techniques appear to be acceptable to people with cystic fibrosis, and do not seem to have associated adverse effects.

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See also

Hum Gene Ther. 2007 Aug;18(8):726-32.

Keywords

Adult; non pharmacological intervention - devices OR physiotherapy; Respiratory Tract Diseases; exercise;