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:** 2007 **Author:** Moss RB

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

Double-blind randomised placebo controlled trial, parallel design, intention-to-treat basis.

Participants

102 CF patients (54 males, 48 females). Mean age 22.6 years, all aged over 12 years. FEV1 >60% predicted.

Interventions

1x10(13) particles tgAAVCF 2 times (30 day interval) or matching placebo, nebulised to lungs.

Outcome measures

Respiratory exacerbations, adverse events, lung function.

Main results

There were no significant differences in spirometric lung function over time, induced sputum biologic markers, or days of antibiotic use in either treatment group. Thus repeated doses of aerosolized tgAAVCF were safe and well tolerated, but did not result in significant improvement in lung function over time. Because gene transfer is the simplest, most basic way to correct the underlying genetic defect that leads to disease in CF, further research is warranted to develop an effective gene transfer agent for the treatment of CF.

Authors' conclusions

Because gene transfer is the simplest, most basic way to correct the underlying genetic defect that leads to disease in CF, further research is warranted to develop an effective gene transfer agent for the treatment of CF.

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See also

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Keywords

Adult; Child; Infection; Inhalation OR nebulised; non pharmacological intervention - genetic& reprod; pharmacological_intervention; placebo; Respiratory Tract Diseases; Respiratory Tract Infections; tgAAVCF; Gene Transfer Techniques;