primary studies - published RCT

A phase II, double-blind, randomized, placebo-controlled clinical trial of tgAAVCF using maxillary sinus delivery in patients with cystic fibrosis with antrostomies.

Code: PM12162817 **Year:** 2002 **Date:** 2002 **Author:** Wagner JA

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

Phase II, double-blind, randomised, placebo-controlled clinical trial

Participants

23 CF patients

Interventions

For each patient, a dose of 100,000 replication units of tgAAVCF was administered to one maxillary sinus, while the contralateral maxillary sinus received a placebo treatment, thereby establishing an inpatient control.

Outcome measures

primary efficacy endpoint (rate of relapse of clinically defined, endoscopically diagnosed recurrent sinusitis); secondary endpoints (sinus transepithelial potential difference [TEPD], histopathology, sinus fluid interleukin [IL]-8 measurements, sinus fluid cytokine IL-10), adverse events, sinus histopathology, serum-neutralizing antibody titer to adeno-associated virus (AAV) capsid protein

Main results

Neither the primary endpoint, nor several secondary endpoints achieved statistical significance when comparing treated to control sinuses within patients. cytokine IL-10 in sinus fluid, was significantly (p

Authors' conclusions

this Phase II trial confirms the safety of tgAAVCF but provides little support of its efficacy in the within-patient controlled sinus study. Various potentially confounding factors are discussed.

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See also

Hum Gene Ther. 2002 Jul 20;13(11):1349-59.

Keywords

Adolescent; Adult; Bacterial Infections; Gene Transfer Techniques; Infection; Instillation- Drug; Intranasal; non pharmacological intervention - genetic& reprod; pharmacological_intervention; placebo; Respiratory Tract Diseases; Respiratory Tract Infections; Virus; tgAAVCF;