

*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.

<http://dx.doi.org/10.1089/104303402760128577>

### **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;