

primary studies - published RCT

## **Gentamicin-induced correction of CFTR function in patients with cystic fibrosis and CFTR stop mutations.**

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

double-blind, placebo-controlled, dose-escalation trial

### **Participants**

12 adults and 12 adolescent patients

### **Interventions**

Single daily escalating doses of AI 75, 150, or 225 mg, or placebo were self-administered using an eFlow Electronic Nebulizer.

### **Outcome measures**

Sputum samples were collected up to 4 hr and blood samples up to 8 hr post-dose.

### **Main results**

AI activity against multiple CF isolates was retained after nebulization via eFlow, and activity was not inhibited by CF sputum. All 12 adult subjects and 11/12 adolescents tolerated all AI doses. One patient had an asymptomatic FEV1 decrease > 20% with the 150 mg dose. Median aztreonam sputum concentrations in adults 10 min after AI 75, 150, and 225 mg were 383, 879, and 985 microg/g, respectively. Median sputum concentrations in adolescents 10 min after AI 75, 150, and 225 mg were 324, 387, and 260 microg/g, respectively. Systemic exposure to AI was low. Plasma pharmacokinetics in adults receiving AI 75 mg were C<sub>max</sub> = 419 ng/g, T<sub>max</sub> = 0.99 hr, t<sub>1/2</sub> = 2.1 hr. Aztreonam concentrations in sputum were at or above the MIC<sub>50</sub> for at least 4 hr post-dose.

### **Authors' conclusions**

These data support the continued development of AI for treatment of pulmonary infections in patients with CF.

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### **See also**

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### **Keywords**

Adolescent; Adult; Anti-Bacterial Agents; Aztreonam; Inhalation OR nebulised; pharmacological\_intervention; Supplementation; Bacterial Infections; Respiratory Tract Infections; Respiratory Tract Diseases; Infection; Pseudomonas aeruginosa; Pseudomonas; Dose-Escalating; Self-Management; Monobactams;