

primary studies - published RCT

## **Repeated nebulisation of non-viral CFTR gene therapy in patients with cystic fibrosis: a randomised, double-blind, placebo-controlled, phase 2b trial.**

**Code:** PM26149841

**Year:** 2015 **Date:** 2020

**Author:** Alton EW

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

Double-blind, randomised controlled trial

### **Participants**

25 patients with *P. aeruginosa* infection admitted to two CF centres for treatment of an acute pulmonary exacerbation

### **Interventions**

Patients) were randomly assigned to receive either 75 mg CaEDTA in Tris-buffered saline or placebo (Tris-buffered saline) nebulised in combination with 250 mg tobramycin twice daily for six weeks followed with four week safety follow-up.

### **Outcome measures**

Primary endpoints were safety, tolerability, and bacterial density of *P. aeruginosa*. A secondary endpoint was lung function.

### **Main results**

The study drug was well tolerated with adverse events comparable in both groups. The mean (SD) reduction in sputum *P. aeruginosa* count (log<sub>10</sub> CFU/g) in the CaEDTA vs placebo group was 2.05 (2.57) vs 0.82 (2.71) at two weeks relative to admission ( $p = 0.39$ ). The mean improvement in ppFEV<sub>1</sub> was 16 vs 5 ( $p = 0.16$ ); 11 vs 2 ( $p = 0.28$ ); and 6 vs 2 percentage points ( $p = 0.47$ ) at two, six, and ten weeks in CaEDTA and placebo groups, respectively.

### **Authors' conclusions**

In this pilot study in CF patients, an increase in the reduction of sputum density of *P. aeruginosa* and an increase in ppFEV<sub>1</sub> was observed in the group of patients who received Tris-CaEDTA added to inhaled tobramycin compared to the group who received inhaled tobramycin alone, although these differences were not statistically significant. The treatment was also shown to be safe.

[http://dx.doi.org/10.1016/S2213-2600\(15\)00245-3](http://dx.doi.org/10.1016/S2213-2600(15)00245-3)

### **See also**

Lancet Respir Med. 2015 Sep;3(9):684-91. doi: 10.1016/S2213-2600(15)00245-3. Epub 2015 Jul 3.

### **Keywords**

Anti-Bacterial Agents; Pseudomonas aeruginosa; Pseudomonas; Tobramycin; Bacterial Infections; Infection; Inhalation OR nebulised; nebuliser; non pharmacological intervention - devices OR physiotherapy; pharmacological\_intervention; Respiratory Tract Diseases; Respiratory Tract Infections; Airway clearance technique; Vibration; Aminoglycosides; oscillating devices; Chest physiotherapy;