primary studies - published RCT

Ataluren (PTC124) induces cystic fibrosis transmembrane conductance regulator protein expression and activity in children with nonsense mutation cystic fibrosis.

Code: PM20622033 **Year:** 2010 **Date:** 2013 **Author:** Sermet-Gaudelus I

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

Randomised controlled trial

Participants

Children with CF (0-18years) and a new isolation of Pa from sputum, cough swab or BAL

Interventions

Children were randomized to treatment with tobramycin inhalation solution for 28days (TIS) or inhaled sodiumcolistimethate (2x2millU/day) plus oral ciprofloxacin (30mg/kg/day) for 3months (CC).

Outcome measures

Airway cultures were taken for 6 consecutive months, then every 3months. The primary outcome was Pa eradication at the end of treatment. Secondary outcome parameters were: time to Pa relapse from end of treatment, total and Pa specific IgG, FEV(1), BMI and Pa status at 2year follow-up.

Main results

58 patients with new Pa isolation were randomized. Their median age was 9years (IQR 4.7-13.1) and their median FEV(1) 98% predicted (IQR 87-107). Eighteen treatments concerned the first Pa isolation 'ever' (TIS: 8; CC: 10). For the remaining, median time since previous Pa was 19months (IQR 9-41). Eradication at end of treatment was similar for both treatments: 26/29 CC and 23/29 in TOBI treated patients (p=0.47). Median time to recurrence of Pa was 9months (95% CI 0.0-19.0) for CC and 5months (95% CI 1.7-8.3) for TIS (p=0.608). After 1year, the 2 groups did not differ in change in total and Pa specific IgG, FEV(1) and BMI. After 2years, 10% of patients had chronic Pa infection.

Authors' conclusions

In children with CF and new Pa infection, inhalation of TIS (28days) or CC (3months) resulted in similar eradication success at the end of treatment (80 and 90% respectively) and similar clinical evolution during the first 2years of follow-up.

http://dx.doi.org/10.1164/rccm.201001-0137OC

See also

American journal of respiratory and critical care medicine YR: 2010 VL: 182 NO: 10

Keywords

Anti-Bacterial Agents; Bacterial Infections; colistimethate; Colistin; Infection; Inhalation OR nebulised; pharmacological_intervention; Pseudomonas aeruginosa; Pseudomonas; Respiratory Tract Diseases; Respiratory Tract Infections; other anti-bacterial agents; Tobramycin; Aminoglycosides; Ciprofloxacin; Quinolones;