

primary studies - published RCT

## Oral cysteamine as an adjunct treatment in cystic fibrosis pulmonary exacerbations: An exploratory randomized clinical trial.

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

Multicentre double-blind randomized clinical trial.

### Participants

Adults experiencing a pulmonary exacerbation of CF being treated with standard care that included aminoglycoside therapy. Eighty nine participants in fifteen US and EU centres were randomized.

### Interventions

Patients were randomized equally to a concomitant 14-day course of placebo, or one of 5 dosing regimens of cysteamine.

### Outcome measures

Outcomes were recorded on days 0, 7, 14 and 21 and included sputum bacterial load and the patient reported outcome measures (PROMs): Chronic Respiratory Infection Symptom Score (CRISS), the Cystic Fibrosis Questionnaire-Revised (CFQ-R); FEV1, blood leukocyte count, and inflammatory markers.

### Main results

78 subjects completed the 14-day treatment period. Cysteamine had no significant effect on sputum bacterial load, however technical difficulties limited interpretation. The most consistent findings were for cysteamine 450mg twice daily that had effects additional to that observed with placebo, with improved symptoms, CRISS additional 9.85 points (95% CI 0.02, 19.7)  $p = 0.05$ , reduced blood leukocyte count by  $2.46 \times 10^9 / l$  (95% CI 0.11, 4.80),  $p = 0.041$  and reduced CRP by geometric mean 2.57 nmol/l (95% CI 0.15, 0.99),  $p = 0.049$ .

### Authors' conclusions

In this exploratory study cysteamine appeared to be safe and well-tolerated. Future pivotal trials investigating the utility of cysteamine in pulmonary exacerbations of CF need to include the cysteamine 450mg doses and CRISS and blood leukocyte count as outcome measures.

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### See also

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### Keywords

cysteamine; Lynovex; Anti-bacterial agents; pharmacological\_intervention; Airway clearance drugs -expectorants- mucolytic-mucociliary-; Exacerbation; Respiratory Tract Infections; Respiratory Tract Diseases; Infection; Bacterial Infections;