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Strategies for newborn screening for cystic fibrosis: A systematic review of health economic evaluations.

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

Randomised controlled trials in people with cystic fibrosis with a pulmonary exacerbation in whom treatment with inhaled antibiotics was compared to placebo, standard treatment or another inhaled antibiotic for between one and four weeks.

List of included studies (5)

Cooper 1985; Schaad 1987; Shatunov 2001; Stephens 1983

Participants

Children and adults with cystic fibrosis who are diagnosed with having a pulmonary exacerbation

Interventions

any inhaled antibiotic, at any dose, using any method of aerosol delivery. Duration of treatment will be between one and four weeks.

Outcome measures

Primary outcomes: QoL, time off work or school, FEV1, FVC, annual change in FEV1

Main results

Five trials with 183 participants are included in the review. Two trials (77 participants) compared inhaled antibiotics alone to intravenous antibiotics alone and three trials (106 participants) compared a combination of inhaled and intravenous antibiotics to intravenous antibiotics alone. Trials were heterogenous in design and two were only available in abstract form. Risk of bias was difficult to assess in most trials but, for four out of five trials, authors judged there to be a high risk from lack of blinding and an unclear risk with regards to randomisation. Results were not fully reported and only limited data were available for analysis. One trial was a cross-over design and we only included data from the first intervention arm. - Inhaled antibiotics alone versus intravenous antibiotics alone: only one trial (18 participants) reported a perceived improvement in lifestyle (quality of life) in both groups (very low certainty evidence). Neither trial reported on time off work or school. Both trials measured lung function, but there was no difference reported between treatment groups (very low certainty evidence). With regards to our secondary outcomes, one trial (18 participants) reported no difference in the need for additional antibiotics and the second trial (59 participants) reported on the time to next exacerbation. In neither case was a difference between treatments identified (both very low certainty evidence). The single trial (18 participants) measuring adverse events and sputum microbiology did not observe any in either treatment group for either outcome (very low certainty evidence). - Inhaled antibiotics plus intravenous antibiotics versus intravenous antibiotics alone: inhaled antibiotics plus intravenous antibiotics may make little or no difference to quality of life compared to intravenous antibiotics alone. None of the trials reported time off work or school. All three trials measured lung function, but found no difference between groups in forced expiratory volume in one second (two trials; 44 participants; very low certainty evidence) or vital capacity (one trial; 62 participants). None of the trials reported on the need for additional antibiotics. Inhaled plus intravenous antibiotics may make little difference to the time to next exacerbation; however, one trial (28 participants) reported on hospital admissions and found no difference between groups. There is likely no difference between groups in adverse events (very low certainty evidence) and one trial (62 participants) reported no difference in the emergence of antibiotic-resistant organisms (very low certainty evidence).

Authors' conclusions

Authors identified only low or very low certainty evidence to judge the effectiveness of inhaled antibiotics for the treatment of pulmonary exacerbations in people with cystic fibrosis. The included trials were not sufficiently powered to achieve their goals. Hence, they are unable to demonstrate whether one treatment was superior to the other or not. Further research is needed to establish whether inhaled tobramycin may be used as an alternative to intravenous tobramycin for some pulmonary exacerbations.

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See also

J Cyst Fibros. 2018 May;17(3):306-315. doi: 10.1016/j.jcf.2018.03.002. Epub 2018 Mar 20.

Keywords

Anti-Bacterial Agents; Inhalation OR nebulised; pharmacological_intervention; Bacterial Infections; Respiratory Tract Infections; Respiratory Tract Diseases; Infection; Exacerbation;