primary studies - published RCT

Impact of Acute Antibiotic Therapy on the Pulmonary Exacerbation Endpoint in Cystic Fibrosis Clinical Trials.

Code: PM23770109 **Year:** 2013 **Date:** 2013 **Author:** Mayer-Hamblett N

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

Randomized, placebo-controlled trial

Participants

260 participants with CF and pulmonary exacerbations (PEs, defined using a priori criteria).

Interventions

Antibiotic therapy. Physician initiated antibiotic therapy (PIT) not meeting the PE endpoint was characterized and its impact on treatment effect assessed.

Outcome measures

Courses of PIT in the absence of a PE (not fulfilling the PE definition).

Main results

40% (104/260) of participants were prescribed 188 courses of PIT in the absence of a PE; 19% (25/129) of placebo and 10% (13/131) of AZ participants received >/=2 courses of PIT and never fulfilled the PE definition (9% difference,95% confidence interval:1%,18%,p=0.04). Accounting for PIT through use of a composite endpoint including time to PE or need for repeated PIT altered treatment effect estimates (a 56% reduction in the event rate comparing AZ to placebo [p

Authors' conclusions

PIT is common in CF and may impact treatment effect estimates. Optimization of the PE endpoint to include meaningful events necessitating treatment may improve our ability to conduct efficient trials by reducing sample size 30-50%, ultimately enabling rapid evaluation of new therapies.

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See also

Contemp Clin Trials. 2013 Jun 13. pii: S1551-7144(13)00096-7. doi: 10.1016/j.cct.2013.06.004.

Keywords

Adolescent; Adult; Anti-Bacterial Agents; Azithromycin; Child; pharmacological_intervention; Bacterial Infections; Respiratory Tract Infections; Respiratory Tract Diseases; Infection; Macrolides; Exacerbation;