primary studies - published RCT

Chest computed tomography outcomes in a randomized clinical trial in cystic fibrosis: Lessons learned from the first ataluren phase 3 study.

Code: PM33141825 **Year:** 2020 **Date:** 2020 **Author:** Tiddens HAWM

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

Phase 3 randomized double blind controlled, trial

Participants

238 people with cystic fibrosis (CF) and at least one nonsense mutation (nmCF)

Interventions

Ataluren. 48 weeks

Outcome measures

Independent scoring systems to characterize structural lung disease. Perth-Rotterdam Annotated Grid Morphometric Analysis for CF (PRAGMA-CF) scoring systems. CF-CT and PRAGMA-CF subscores were expressed as %maximal score and %total lung volume, respectively.

Main results

PRAGMA-CF subscores %Disease (p = 0.008) and %Mucus Plugging (p = 0.029) progressed over 48 weeks. CF-CT subscores did not show progression. There was no difference in progression of structural lung disease between treatment arm and placebo independent of tobramycin use.

Authors' conclusions

PRAGMA-CF Chest CT scores can be used as an outcome measure to study the effect of potential disease modifying drugs in CF on lung structure.

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See also

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Keywords

PTC124; Ataluren; CFTR Modulators; pharmacological_intervention;