primary studies - published RCT

Efficacy and safety of ataluren in patients with nonsense-mutation cystic fibrosis not receiving chronic inhaled aminoglycosides: The international, randomized, double-blind, placebo-controlled Ataluren Confirmatory Trial in Cystic Fibrosis (ACT CF).

Code: PM31983658 **Year:** 2020 **Date:** 2020 **Author:** Konstan MW

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

International, randomized, double-blind, placebo-controlled Trial

Participants

Subjects with nonsense-mutation CF (aged >/=6 years; percent predicted (pp) FEV1 >/=40 and

Interventions

Subjects were randomly assigned in double-blinded fashion to receive oral ataluren or matching placebo thrice daily for 48 weeks.

Outcome measures

The primary endpoint was absolute change in average ppFEV1 from baseline to the average of Weeks 40 and 48.

Main results

279 subjects were enrolled; 138 subjects in the ataluren arm and 136 in the placebo arm were evaluable for efficacy. Absolute ppFEV1 change from baseline did not differ significantly between the ataluren and placebo groups at Week 40 (-0.8 vs -1.8) or Week 48 (-1.7 vs -2.4). Average ppFEV1 treatment difference from baseline to Weeks 40 and 48 was 0.6 (95% CI -1.3, 2.5; p = 0.54). Pulmonary exacerbation rate per 48 weeks was not significantly different (ataluren 0.95 vs placebo 1.13; rate ratio p = 0.40). Safety was similar between groups. No life-threatening adverse events or deaths were reported.

Authors' conclusions

Neither ppFEV1 change nor pulmonary exacerbation rate over 48 weeks were statistically different between ataluren and placebo groups. Development of a nonsense-mutation CF therapy remains elusive.

http://dx.doi.org/10.1016/j.jcf.2020.01.007

See also

J Cyst Fibros. 2020 Jan 23. pii: S1569-1993(20)30030-8. doi: 10.1016/j.jcf.2020.01.007.

Keywords

PTC124; Ataluren; CFTR Modulators; pharmacological_intervention;