primary studies - published RCT

Safety and efficacy of recombinant alpha(1)-antitrypsin therapy in cystic fibrosis.

Code: PM16372352 **Year:** 2006 **Date:** 2006 **Author:** Martin SL

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

prospective, double-blinded, randomized, placebo-controlled phase II trial

Participants

39 CF patients

Interventions

Subjects were randomized to receive nebulized treatment once a day for 4 weeks, followed by 2-4 weeks with no study treatment, and then a 2-week rechallenge phase

Outcome measures

Examine the effect of rAAT (500, 250, and 125 mg) on sputum NE activity. Sputum myeloperoxidase (MPO), interleukin-8, tumor necrosis factor receptors, sputum and plasma NE/AAT complexes, and safety parameters were measured

Main results

Trends toward a reduction in NE activity were observed in patients treated with 500 mg and 250 mg of rAAT compared to placebo. Sputum NE/AAT complex and MPO levels were lower on rAAT compared to placebo. No major adverse events and, in particular, no allergic reactions to rAAT were observed. Although significant differences between rAAT and placebo for sputum NE activity were not observed, some improvements were found for secondary efficacy variables.

Authors' conclusions

This study demonstrated that nebulized rAAT is safe and well-tolerated, but has a limited effect on NE activity and other markers of inflammation.

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See also

Pediatr Pulmonol. 2006 Feb;41(2):177-83.

Keywords

Adolescent; Adult; Aged; Inhalation OR nebulised; nebuliser; non pharmacological intervention - devices OR physiotherapy; pharmacological_intervention; Recombinant Proteins; Respiratory System Agents; alpha1-anti-trypsin; Anti-Inflammatory Agents; Anti-Inflammatory Agents - excl Steroids;