primary studies - published, non RCT

Emulated trial investigating effects of multiple treatments: estimating combined effects of mucoactive nebulisers in cystic fibrosis using registry data.

Code: PM37451864 **Year:** 2023 **Date:** **Author:** Granger E

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

Using UK CF Registry data from 2007 to 2018, authors emulated a target trial.

Participants

People with CF aged 6 years

Interventions

Dornase alfa (DNase) and hypertonic saline (HS). The effects of DNase and HS used alone have been studied in randomised trials, but their effects in combination have not. This study investigates whether, for people already prescribed DNase, adding HS has additional benefit for lung function or use of intravenous antibiotics.

Outcome measures

Forced expiratory volume in 1 s (FEV(1)%) predicted

Main results

4498 individuals were included. At baseline, average age and forced expiratory volume in 1 s (FEV(1)%) predicted were 21.1 years and 69.7 respectively. During first year of follow-up, 3799 individuals were prescribed DNase alone; 426 added HS; 57 switched to HS alone and 216 were prescribed neither. We found no evidence that adding HS improved FEV(1)% at 1-5 years, or use of intravenous antibiotics at 1-4 years, compared with DNase alone.

Authors' conclusions

For individuals with CF prescribed DNase, we found no evidence that adding HS had an effect on FEV(1)% or prescription of intravenous antibiotics. Our study illustrates the emulated target trial approach using CF Registry data.

http://dx.doi.org/10.1136/thorax-2023-220031

See also

Thorax. 2023 Jul 14:thorax-2023-220031. doi: 10.1136/thorax-2023-220031.

Keywords

hydration; Hypertonic Solutions; Inhalation OR nebulised; nebuliser; non pharmacological intervention - devices OR physiotherapy; pharmacological_intervention; Airway clearance drugs -expectorants- mucolytic- mucociliary-; Respiratory System Agents; Deoxyribonuclease; Dornase alpha; Pulmozyme;