primary studies - published RCT

CFTR activity is enhanced by the novel corrector GLPG2222, given with and without ivacaftor in two randomized trials.

Code: PM31056441 **Year:** 2019 **Date:** **Author:** Bell SC

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

The SIMPLIFY study included two parallel, multicentre, open-label, randomised, controlled, non-inferiority trials at 80 participating clinics across the USA in the Cystic Fibrosis Therapeutics Development Network.

Participants

Participants with well-preserved pulmonary function taking elexacaftor + tezacaftor + ivacaftor (ETI)

Interventions

People taking ETI continued or discontinued (1) dornase alfa or (2) hypertonic saline

Outcome measures

The primary outcomes were differences in costs of outpatient prescription drugs among those who continued vs discontinued dornase alfa and, separately, hypertonic saline. To estimate the annual cost savings if the population of people with CF taking ETI used these medications only intermittently, authors multiplied the proportion of people in MarketScan with CF diagnoses who were taking each of these medications by the median cost savings per year and subtracted the cost of "rescue" use.

Main results

A total of 392 participants from the dornase alfa trial and 273 from the hypertonic saline trial were included in analyses. The adjusted difference in median medication costs was not significant for the hypertonic saline trial, but we observed a significantly decreased 6-week cost of medications in the dornase alfa trial (adjusted median difference in costs between discontinue and continue of $5,860 (95% CI = $4,870-$6,850); P

Authors' conclusions

Although the costs of dornase alfa and hypertonic saline are smaller compared with ETI, reduction in use would lead to substantial prescription drug cost savings and reduce the treatment burden. However, individual benefits of these therapies should be considered, and decisions regarding changes in therapy remain an important discussion between people with CF and their providers.

http://dx.doi.org/10.1016/j.jcf.2019.04.014

See also

J Cyst Fibros. 2019 Sep;18(5):700-707. doi: 10.1016/j.jcf.2019.04.014. Epub 2019 May 3.

Keywords

Adolescent; Child; Deoxyribonuclease; Drug Administration Schedule; Airway clearance drugs -expectorants- mucolytic- mucociliary-; hydration; Hypertonic Solutions; Inhalation OR nebulised; nebuliser; non pharmacological intervention - devices OR physiotherapy; pharmacological_intervention; Recombinant Proteins; Respiratory System Agents; Dornase alpha; Pulmozyme; Aminophenols; CFTR Modulators; Genetic Predisposition to Disease;