primary studies - published, non RCT

Growth in Prepubertal Children With Cystic Fibrosis Treated With Ivacaftor.

Code: PM28143919 **Year:** 2017 **Date:** 2017 **Author:** Stalvey MS

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

Post hoc analysis (2 clinical trials, the longitudinal-observation GOAL study and the placebo-controlled ENVISION study)

Participants

83 children (aged 6-11 years) enrolled in 2 clinical trials, the longitudinal-observation GOAL study and the placebo-controlled ENVISION study

Interventions

Ivacaftor

Outcome measures

Height and weight z scores and height and weight growth velocities (GVs).

Main results

In ivacaftor-treated children in GOAL, height and weight z scores increased significantly from baseline to 6 months (increases of 0.1 [P

Authors' conclusions

Ivacaftor treatment in prepubescent children may help to address short stature and altered GV in children with CF; results from these analyses support the existence of an intrinsic defect in the growth of children with CF that may be ameliorated by CFTR modulation.

http://dx.doi.org/10.1542/peds.2016-2522

See also

Pediatrics. 2017 Feb;139(2). pii: e20162522. doi: 10.1542/peds.2016-2522.

Keywords

Child; Hormones; pharmacological_intervention; Recombinant Proteins; Growth Hormone;