Growth in Prepubertal Children With Cystic Fibrosis Treated With Ivacaftor.

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.

See also

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