

#### primary studies - published, non RCT

# Growth in Prepubertal Children With Cystic Fibrosis Treated With Ivacaftor.

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# 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;