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*primary studies - published RCT*

## **Efficacy and Safety of Ivacaftor in Patients Aged 6 to 11 Years with Cystic Fibrosis with a G551D Mutation.**

**Code:** PM23590265

**Year:** 2013 **Date:** 2013

**Author:** Davies JC

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

Randomized, double-blind, placebo-controlled trial

### **Participants**

Patients with CF aged 6-11 years with a G551D-CFTR mutation on at least one allele.

### **Interventions**

Patients were randomly assigned to receive ivacaftor administered orally at 150 mg (n=26) or placebo (n=26) every 12 hours for 48 weeks in addition to existing prescribed cystic fibrosis therapies.

### **Outcome measures**

Forced expiratory volume in 1 second (FEV1); concentration of sweat chloride; incidence of adverse events

### **Main results**

Despite near normal mean baseline values in forced expiratory volume in 1 second (FEV1), patients receiving ivacaftor had a significant increase in percent predicted FEV1 from baseline through Week 24 versus placebo group (treatment effect: 12.5 percentage points; P

### **Authors' conclusions**

In patients who are younger and healthier than those in previously studied populations, ivacaftor demonstrated a meaningful improvement in pulmonary function, weight, and CFTR activity compared with placebo.

<http://dx.doi.org/10.1164/rccm.201301-0153OC>

### **See also**

Am J Respir Crit Care Med. 2013 Apr 3.

### **Keywords**

Child; Aminophenols; CFTR Modulators; Genetic Predisposition to Disease; pharmacological\_intervention; VX-770; ivacaftor; G551D-CFTR;