# Cystic Fibrosis Research News

**Title:**
Changes in nutrition and growth status in young children in the first 12 weeks of ivacaftor therapy

**Lay Title:**
Ivacaftor impacts nutrition and growth status in 4 to 24 month old children within the first 12 weeks

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**What was your research question?**
What is the impact of cystic fibrosis drug, ivacaftor, on nutrition, growth and other health outcomes in children 4 to 24 months of age after 12 weeks of therapy.

**Why is this important?**
Cystic fibrosis (CF) often causes problems with growth and development. Ivacaftor improves body weight and BMI in older children and adults, in addition to improved lung function and are important clinical outcomes in CF care. Ivacaftor also improves use of calories, gut inflammation, dietary fat absorption. However, the impact of ivacaftor on non-lung outcomes had not been explored in young children. This group of children is of particular importance as they are rapidly growing.

**What did you do?**
Children 4 to 24 months who were approved to start ivacaftor therapy were recruited from US and Canadian CF Centres. Weight, height, skinfolds, diet, sleeping energy expenditure (SEE), nutrition biomarkers, pancreatic status, serum and faecal calprotectin, serum bile acids, plasma fatty acids were measured before starting ivacaftor and after six and 12 weeks after starting the drug. Changes from baseline at 6 and 12 weeks were examined using mixed effects linear regression modelling.

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## What did you find?

Children with CF gained weight similar to children of the same age and sex who are otherwise healthy, which is an important and exciting finding for young children who often experience challenges gaining weight and growing taller. Further, these children required a similar calories from food to maintain essential body functions as healthy children. Therefore, increased energy needs are not a barrier for young children with CF to experience healthy growth. There were also many other favourable changes in nutrition outcomes, such as blood urea nitrogen and pre-albumin, and novel information on bile acid metabolism.

## What does this mean and reasons for caution?

This study provides efficacy and safety results on weight gain, nutritional and other biomarkers in very young children on ivacaftor. In the context of the first 12 weeks of ivacaftor initiation in children 4 to 20 months of age at enrolment, therapy supported significant weight gain and clinically meaningful improvements in nutritional laboratory measures. However, this study is limited by a relatively small sample size. Several outcomes approached statistical significance; other outcomes may not have reached significance due to the small number of subjects.

## What's next?

Future studies might aim to examine nutritional, growth and markers of health aside from lung function over a longer period of time and measure body composition to understand weight gain in muscle and fat stores in young children as CFTR modulator treatment is available to young children across all mutation classes.

## Original manuscript citation in PubMed

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