



Effective Health Care Program

# Human Growth Hormone for Children With Cystic Fibrosis

A Review of the Research for Parents and Caregivers



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## About This Guide

### **Who is this guide for?**

This guide is for anyone who takes care of a child or teen with cystic fibrosis (CF) and helps make decisions about treatment. You may have heard about using human growth hormone (HGH) for children with CF and want to know more about the research.

### **Why review the research on human growth hormone?**

While HGH does not directly treat CF, researchers have wanted to know whether growing taller or gaining more weight by taking HGH could improve breathing or other aspects of health.

### **What is not covered in this guide?**

Many treatments and therapies for CF are not covered in this guide, including other treatments that help children with CF to gain weight. Also, this guide does not cover the use of HGH for adults with CF.

### **Where does the information in this guide come from?**

The information comes from a review of 79 clinical studies. Independent researchers, funded by the Agency for Healthcare Research and Quality (AHRQ), reviewed the evidence about using HGH for children with CF. The research questions and the results of the report were subject to expert input, peer review, and public comment. You can read the full report at <http://www.effectivehealthcare.ahrq.gov/hgh.cfm>.

## Understanding Cystic Fibrosis

### What is cystic fibrosis?

- CF is an inherited disease that affects about 30,000 children and adults in the United States.
- CF is seen most often in children who are white, but it can occur in children of all races and ethnic backgrounds.
- CF occurs when children inherit an abnormal gene from each parent, even though each of the parents may not have the disease.
- The abnormal gene causes the child's body to make thick, sticky mucus. This mucus can:
  - Plug the lungs, making it difficult to breathe.
  - Obstruct the pancreas or other organs that help digest food.
  - Trap germs that cause infections.

### How does cystic fibrosis affect growth?

CF can keep children from getting all the calories and nutrients they need from food. Because of this, many children with CF fall below the normal height and weight averages for their age.

Some research suggests that below-normal height and weight may increase children's risk for other problems related to CF and may keep them from living a longer life.

## Understanding Human Growth Hormone

### What is human growth hormone?

HGH is one of many hormones made by your “pituitary” (pronounced pi-TOO-it-air-ee) gland, which is located right under the brain. Hormones are chemical messengers that send signals to the cells in your body. Some messages tell your cells how to use food or make more cells. Growth hormone sends signals that tell the cells in the muscles, bones, and organs of the body to grow.

### How is human growth hormone used as a medicine?

For use as a medicine, human growth hormone is made in a laboratory. This form is called “somatropin” (pronounced so-mah-TROW-pin). It is also called “recombinant human growth hormone” or rhGH.

Somatropin is given as a shot at home every day. It is approved by the United States Food and Drug Administration (FDA) for growth problems in children.



## Understanding the Research About Growth Hormone

### Are there benefits?

The available research cannot say if taking somatropin can help improve the quality or length of life for children or teens with CF.

Children who had somatropin added to their other treatments for CF for 6 to 12 months had some small changes. These children:

- Stayed one to two fewer nights in the hospital per year than children who did not take somatropin.
- Grew a little more than 1 inch taller and gained about 3 pounds more than children who did not take somatropin.
- Showed small increases in several measurements of their lung function, but not in the measure of lung function that researchers say is associated with the length of life.

### Are there risks?

- Very few children with CF who have taken somatropin in studies had to stop because of side effects, such as a reaction from the shot.
- People with CF are at increased risk for developing CF-related diabetes. Researchers cannot say if somatropin increases a child's risk of getting CF-related diabetes.
- Adults with CF are at an increased risk of developing certain types of digestive or liver cancer. Researchers cannot say if people with CF who have used somatropin have a higher risk of developing cancers.

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## What are the costs?

The cost to you for somatropin depends on several things, including:

- Your health insurance.
- The amount (dose) of the medicine and how often a child needs to take it.
- The actual cost of the medicine to the hospital or pharmacy.

Insurance may not cover the cost of somatropin for children with CF. Human growth hormone treatment is expensive. The cost to buy enough somatropin to treat a typical adolescent could be tens of thousands of dollars each year.





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## Source

The information in this guide comes from the report *Effectiveness of Recombinant Human Growth Hormone (rhGH) in the Treatment of Patients With Cystic Fibrosis*. It was produced by the University of Connecticut/Hartford Hospital Evidence-based Practice Center through funding by the Agency for Healthcare Research and Quality (AHRQ). For a copy of the report or for more information about AHRQ and the Effective Health Care Program, go to <http://www.effectivehealthcare.ahrq.gov/hgh.cfm>.

This summary guide was prepared by the John M. Eisenberg Center for Clinical Decisions and Communications Science at Baylor College of Medicine, Houston, TX. Parents and caregivers of children with cystic fibrosis and adults with cystic fibrosis helped develop this guide.

