

**Guidance on  
the use of  
human growth  
hormone  
(somatropin) in  
children with  
growth failure**

Further information about NICE and the full guidance on the use of human growth hormone (somatropin) in children with growth failure that has been issued to the NHS are available on the NICE website at [www.nice.org.uk](http://www.nice.org.uk). The full guidance can also be requested by calling 0870 1555 455 and quoting reference number N0095.

If you have access to the Internet and would like to find out more about growth hormone disorders and how they are treated, visit the NHS Direct website: [www.nhsdirect.nhs.uk](http://www.nhsdirect.nhs.uk). If you would like to speak to NHS Direct please call them on 08 45 46 47.

## **National Institute for Clinical Excellence**

11 Strand  
London  
WC2N 5HR

Web: [www.nice.org.uk](http://www.nice.org.uk)

ISBN: 1-84257-185-0  
Published by the National  
Institute for Clinical Excellence  
May 2002

---

## What is NICE guidance?

The National Institute for Clinical Excellence (NICE) is part of the NHS. It produces guidance on the use of medicines, medical equipment and clinical procedures for people working in the NHS in England and Wales, and for patients and their carers.

To produce this guidance, NICE looks at how well the medicine, equipment or procedure works and also how well it works in relation to how much it costs. This process is called an appraisal. The appraisal process involves the manufacturer of the medicine or equipment for which guidance is being produced, and the organisations that represent the healthcare professionals, patients and carers who will be affected by the guidance. Each appraisal takes about 12 months to complete.

---

## What is this guidance about?

NICE has looked at the evidence available on the use of biosynthesised human growth hormone, which is also called somatropin, in children who are not growing normally – they have ‘growth failure’.

---

## What is human growth hormone?

Human growth hormone is a hormone produced by the pituitary, which is a small gland inside the head. Growth hormone is essential for growth in children. It also has important effects on how the body deals with protein,

fats and carbohydrates, not only during childhood, but also throughout adult life.

Some children do not produce enough growth hormone or do not respond as expected to growth hormone, and so they do not grow as they should. They are much shorter than would be expected for their age.

Children who have 'growth hormone deficiency' do not have enough growth hormone. Sometimes it is known what is causing the lack of growth hormone – for example, there is a problem with the pituitary gland. But for 5 to 7 out of every 10 children with growth hormone deficiency, the cause is not known.

Failure to grow normally is also a key feature of several other medical conditions. The most common of these are reduction in kidney function (known as 'chronic renal insufficiency') and conditions known as Turner syndrome (which only affects girls) and Prader-Willi syndrome.

Somatropin is biosynthetic, or laboratory produced, human growth hormone. It is the same chemically as the growth hormone produced by the body. Somatropin is made by a process known as genetic engineering.

---

What is somatropin and how does it work?

Somatropin is given to increase growth in children who have growth hormone deficiency, Turner syndrome, chronic renal insufficiency or Prader-Willi syndrome. For children with Prader-Willi syndrome, growth hormone treatment is also given to improve body composition – children with Prader-Willi syndrome often have too much body fat and growth hormone can help to correct this.

---

### What has NICE recommended ?

NICE has made the following recommendations about the use of human growth hormone (somatropin) to treat children with growth hormone deficiency, Turner syndrome, chronic renal insufficiency or Prader-Willi syndrome. Note that at the time this guidance was issued, somatropin was not licensed to treat children who are not growing despite normal levels of growth hormone because of another unknown reason, and so this guidance does not deal with that use of somatropin.

- Human growth hormone (somatropin) treatment is recommended for children who have growth hormone deficiency. To help diagnose growth hormone deficiency the doctor should take measurements of height and look at the results of

radiological investigations and tests to measure the amount of GH in the child's blood.

- Growth hormone treatment is recommended for girls with Turner syndrome. For the treatment to work as well as possible, they should be diagnosed and start treatment at the earliest age possible and they should be given oestrogen treatment at the right time to stimulate puberty.
- Growth hormone treatment is recommended for children with chronic renal insufficiency who have not yet reached puberty. Before giving growth hormone treatment the doctor should make sure that the child's nutrition (food intake) and metabolism (chemical processes inside the body) are as good as possible. Treatment with steroids should be at the lowest level possible.
- Growth hormone treatment is recommended for treating children with Prader-Willi syndrome.
- A paediatrician (a doctor who specialises in treating children) who is an expert in treating children with a growth hormone disorder should

always be responsible for starting and checking the progress of growth hormone treatment.

- Usually, growth hormone treatment should be stopped after the first year if the extra height gain is not at least half the height gain in the year before treatment began. For a child who continues treatment after the first year, treatment should be stopped, after discussion with the child and carer, when the expected final height has nearly been reached, or when the child has grown less than 2 cm in 1 year. Alternatively, treatment should be continued until the person can be seen by a doctor who specialises in treating adults with a hormone disorder (an adult endocrinologist).
- The doctor who is treating a child with Prader-Willi syndrome should consider the amount of fat in the body as well as height when considering when to stop treatment with growth hormone. This is because children with Prader-Willi syndrome often have too much body fat.

- The doctor should consider stopping treatment with growth hormone before a child has reached final height if the child is not taking the treatment regularly.
- If a child with chronic renal insufficiency has a kidney transplant, growth hormone treatment should be stopped. It should not usually be started again until at least 1 year after the transplant. This is so that doctors can see whether the child's growth catches up after the transplant.

---

What should I do?

If you are child with a growth hormone disorder, you can discuss this guidance with the doctor at your next hospital appointment. If you are the parent or carer of a child with a growth hormone disorder you should discuss this guidance with the doctor at the next hospital appointment.

---

Will NICE review its guidance?

Yes. This guidance will be reviewed in June 2005.

