

## News release

# NICE recommends somatropin to treat growth failure in children

Final guidance published today (26 May) by NICE recommends somatropin (a synthetic form of the human growth hormone) as a treatment option for two growth disorders in children: short stature homeobox-containing gene (SHOX) deficiency<sup>1</sup>, and for those who were smaller<sup>2</sup> than expected at birth and whose growth has not become normal by the age of four or later.

The guidance, which updates a previous NICE appraisal from 2002, continues to recommend somatropin for children with growth hormone deficiency; Turner syndrome<sup>3</sup>; chronic renal insufficiency (CRI)<sup>4</sup> and Prader-Willi syndrome<sup>5</sup>. In children with Prader-Willi syndrome, somatropin helps to improve body composition (body fat levels and body mass index) as well as encouraging growth. The pituitary gland normally produces the human growth hormone, but if this process is disturbed in some way, a child may not grow sufficiently. In the conditions covered by this guidance, somatropin can help the body to grow.

There are seven versions of somatropin licensed for use in England, and the choice of product should be made on a case-by-case basis after informed discussion between clinician and patient and/or their carer about the advantages and disadvantages of the products available. If, after that discussion, more than one product is suitable, treatment should be started with the least expensive product. The guidance also makes recommendations for stopping treatment with somatropin

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<sup>1</sup> Short stature homeobox gene or SHOX is a gene on the X and Y chromosomes and plays a role in bone growth. SHOX deficiency can cause growth problems in people with conditions such as Turner syndrome.

<sup>2</sup> The international consensus definition of 'small for gestational age' (SGA) is a length or weight at birth that is 2 standard deviations below (< -2 SD) the population average for birth or weight.

<sup>3</sup> Turner syndrome is a genetic condition that affects girls if they do not have the usual pair of two X chromosomes. Those who have this condition are usually shorter than average, and infertile due to early loss of ovarian function.

<sup>4</sup> Chronic renal insufficiency or CRI is sometimes known as renal failure or kidney failure, and it occurs when the kidneys fail to function adequately. There are two forms: acute (acute kidney injury) and chronic (chronic kidney disease).

<sup>5</sup> Prader-Willi syndrome is a genetic disorder caused by an abnormality of chromosome 15 (one of the 23 pairs of chromosomes in humans). Characteristics include short stature, obesity and obesity – related diseases, and behavioural problems.

once the child reaches, or is approaching their final height, or if the treatment is not working as expected.

**Dr Carole Longson, NICE Health Technology Evaluation Centre Director said:**

“We are pleased to recommend somatropin as a clinically and cost effective treatment option for some children with these growth problems. Somatropin is at present the only active treatment for children who are not growing normally, and using it can add as much as 11cm to a child’s height, depending on the underlying condition. This final decision will be welcome news to those who are affected”.

**This final guidance now replaces local recommendations across the country; the NHS has three months to start implementing this new guidance.**

**Ends**

**Notes to Editors**

**About the guidance**

- The final guidance can be found on the NICE website at <http://www.nice.org.uk/guidance/TA188>
- In the UK, seven preparations of somatropin are available:
  1. Genotropin, Pfizer;
  2. Humatrope, Lilly;
  3. Norditropin SimpleXx, Novo Nordisk;
  4. NutropinAq, Ipsen;
  5. Omnitrope, Sandoz;
  6. Saizen, Merck Serono;
  7. Zomacton, Ferring.
- Each product is produced by recombinant DNA technology and has a sequence identical to the human growth hormone produced by the pituitary gland. Not all these technologies are licensed for all the indications covered by this guidance
- The cost of treatment with somatropin depends on the dose, which is determined by the weight or body surface area of the child as well as by the indication for growth hormone treatment. The price of the seven different somatropin products ranges from £18.00 per mg to £23.39 per mg (BNF edition 58). Costs may vary in different settings because of negotiated procurement discounts.
- The final guidance recommends that treatment with somatropin should be stopped if:

- growth rate increases by less than 50% from baseline in the first year of treatment.
- final height is approached and growth rate is less than 2 cm total growth in one year.
- there are insurmountable problems with adherence to the treatment.
- final height is attained.
- In Prader–Willi syndrome, evaluation of the response to treatment should also consider changes in body composition.

The decision to stop treatment should be made in consultation with the patient and/or carers either by:

- a paediatrician with specialist expertise in managing growth hormone disorders in children, or
- an adult endocrinologist, if care of the patient has been transferred from paediatric to adult services.

## About NICE

- The National Institute for Health and Clinical Excellence (NICE) is the independent organisation responsible for providing national guidance on the promotion of good health and the prevention and treatment of ill health.
- NICE produces guidance in three areas of health:
  1. **public health** – guidance on the promotion of good health and the prevention of ill health for those working in the NHS, local authorities and the wider public and voluntary sector
  2. **health technologies** – guidance on the use of new and existing medicines, treatments and procedures within the NHS
  3. **clinical practice** – guidance on the appropriate treatment and care of people with specific diseases and conditions within the NHS