NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE
Health Technology Evaluation

Somapacitan for treating growth hormone deficiency in children

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of somapacitan within its marketing authorisation for treating children with growth hormone deficiency.

Background

Human growth hormone is produced by the anterior pituitary gland. The synthetic form is called somatropin (recombinant human growth hormone). Human growth hormone is essential for normal growth in children. It increases growth by a direct action on the growth plates (the area between the epiphysis and the diaphysis within which bone growth occurs) and by production of insulin-like growth factors (mainly in the liver). Growth hormone also has important effects on the metabolism of proteins, lipids and carbohydrates, not only during childhood, but also throughout adult life.

Growth hormone deficiency occurs when the pituitary gland does not produce enough human growth hormone and is the most common endocrine cause of short stature. Growth failure in children can also be a result of growth hormone deficiency, but also occurs in children with Turner syndrome, chronic renal insufficiency (CRI), short stature homeobox-containing gene (SHOX) deficiency, and in children born small for gestational age.Growth hormone deficiency may also be associated with deficiencies in several pituitary hormones arising from hypopituitarism, tumours in the central nervous system, cranial irradiation or other physiological causes.

The prevalence of growth hormone deficiency is estimated to be between 1 in 3500 and 1 in 4000 children.[1] In about half of the children with growth hormone deficiency (50%), the cause is unknown (idiopathic growth hormone deficiency).[1]

NICE Technology appraisal guidance 188 recommends somatropin (recombinant human growth hormone) as a treatment option for children with growth hormone deficiency. It is administered as a daily subcutaneous injection. The place of somatropin in the treatment pathway depends on the child's particular condition, age at diagnosis and the licensed indications of each of the seven somatropin preparations used in UK practice.

The technology

Somapacitan (Sogroya, Nova Nordisk) does not currently have a marketing authorisation in the UK for the treatment of growth hormone deficiency in children. It has been studied in clinical trials compared with somatropin in children aged between 2.5 and 11 years.

Intervention	Somapacitan
Population	Children and adolescents from 2.5 years of age with growth hormone deficiency
Subgroups	If the evidence allows, the appraisal should consider the transition of care from paediatric to adult endocrine services of young people whose linear growth is not complete.
Comparators	• Somatropin • Somatrogon (subject to NICE evaluation) • Management strategies without human growth hormone
Outcomes	The outcome measures to be considered include: • annual height velocity • height standard deviation score-height relative to the distribution of height in children of the same chronological age • body composition, and biochemical and metabolic markers. • change in bone maturation • adverse effects of treatment • health-related quality of life.
Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. As the technology is likely to provide similar or greater health benefits at similar or lower cost than technologies recommended in published NICE technology appraisal guidance for the same indication, a cost comparison may be carried out. The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared. Costs will be considered from an NHS and Personal Social Services perspective. The availability and cost of biosimilar and generic products should be taken into account.

Other considerations	Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.
Related NICE recommendations	**Related Technology Appraisals:** Human growth hormone (somatropin) for the treatment of growth failure in children (2010). NICE Technology appraisal guidance 188. This guidance will be reviewed if there is new evidence. Human growth hormone (somatropin) in adults with growth hormone deficiency (2003). NICE technology appraisal guidance 64. This guidance will be reviewed if there is new evidence. **Related technology appraisals in development:** Somatrogon for treating growth disturbance in children and young people aged 3 and over. NICE technology appraisal ID5086. Publication date TBC. **Related Guidelines:** Faltering growth: recognition and management of faltering growth in children (2017). NICE guideline 75. Updated 2021. Review date TBC. **Related Quality Standards:** Faltering growth (2020). NICE quality standard 197.
Related National Policy	The NHS Long Term Plan (2019) NHS Long Term Plan NHS England (2018) NHS manual for prescribed specialist services (2018/2019). Chapter 109. Specialist endocrinology and diabetes services for children (Endocrinology: complex growth problems including Turner syndrome and growth hormone deficiency; puberty disorders including precocious, delayed or absent puberty).

Questions for consultation

Do the interventions listed as comparators reflect the options available in the NHS?

Where do you consider somapacitan will fit into the existing care pathway for children with growth hormone deficiency?

Do you consider that the use of somapacitan can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected

characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which Somapacitan will be licensed;
- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;
- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

NICE is considering evaluating this technology through its cost comparison evaluation process.

Please provide comments on the appropriateness of appraising this topic through this process.

(Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-tehnology-appraisal-guidance/changes-to-health-technology-evaluation).

Technologies can be evaluated through the cost-comparison process if they are expected to provide similar or greater health benefits, at a similar or lower cost, compared with technologies that have been previously recommended (as an option) in published NICE guidance for the same indication. Companies can propose cost-comparison topics to NICE at any stage during topic selection and scoping. NICE will route technologies for evaluation through the cost-comparison process if it is agreed during scoping that the process is an appropriate route to establish the clinical and cost effectiveness of the technology.

NICE's health technology evaluations: the manual states the methods to be used where a cost comparison case is made.

- Is the technology likely to be similar in its clinical effectiveness and resource use to any of the comparators? Or in what way is it different to the comparators?
- Will the intervention be used in the same place in the treatment pathway as the comparator(s)? Have there been any major changes to the treatment pathway recently? If so, please describe.
- Will the intervention be used to treat the same population as the comparator(s)?
- Overall in the technology likely to offer similar or improved health benefits compared with the comparators?
- Would it be appropriate to use the cost-comparison methodology for this topic?

References

1. National Institute for Health and Care Excellence. Human growth hormone (somatropin) for the treatment of growth failure in children (2010). Accessed June 2022.