NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Somatrogon for treating growth disturbance in children and young people aged 3 and over

Final scope

Remit/evaluation objective

To appraise the clinical and cost effectiveness of somatrogon within its marketing authorisation for treating children and adolescents from 3 years of age with growth disturbance due to insufficient secretion of growth hormone.

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]

Somatropin (recombinant human growth hormone) is currently the only active pharmacological treatment option for growth failure in 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

Somatrogon (Ngenla®, Pfizer) is a long acting, once weekly subcutaneous injection of recombinant modified human growth hormone, that consists of naturally occurring carboxy-terminal peptide. The fusion of sialylated and glycosylated carboxy-terminal peptide to somatropin extends its half-life by decreasing clearance and increasing circulatory half-life allowing for once weekly injections.

Somatrogon currently has a market authorisation in the UK for the treatment of children and adolescents from 3 years of age with growth disturbance due to insufficient secretion of growth hormone.

Intervention(s)	Somatrogon
Population(s)	Children and adolescents from 3 years of age with growth disturbance due to insufficient secretion of growth hormone
Comparators	• Recombinant daily human growth hormone (somatropin) • 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. If 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 of any commercial arrangements for the intervention, comparator and subsequent treatment technologies will be taken into account. The availability and cost of biosimilar and generic products should be taken into account.

Other considerations	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. 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 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 (2017). NHS Medicines for Children's Policy NHS England (2018/2019). 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). NHS England (2013). E03/S/e 2013/14 NHS standard contract paediatric medicine: endocrinology & diabetes (paediatric endocrinology is concerned with the diagnosis and management of children and young people with hormonal disorders (including growth and bone problems). Department of Health and Social Care (2016) NHS outcomes framework 2016 to 2017

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.