



Royal College of Paediatrics and Child Health

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RCPCH response to NICE consultation:

Appraisal Consultation Document (ACD) and evaluation report: Human growth hormone (somatropin) for the treatment of growth failure in children (review of NICE technology appraisal guidance 42)

Dear Mr Powell

Thank you for inviting the Royal College of Paediatrics and Child Health to comment on the NICE appraisal consultation document for human growth hormone (somatropin) for the treatment of growth failure in children. Please find our response below.

With thanks to:

- [REDACTED], [REDACTED]

Has all of the relevant evidence been taken into account?

The College is not aware of further relevant published material which should be taken into account. However, we note that much of the evidence cited for the appraisal document was of rather poor quality. There were two main areas of difficulty:

1. The studies used to appraise the use of human growth hormone (HGH) in small for gestation age (SGA) infants used HGH in doses which exceeded UK licensed doses. Clinical experience suggests that higher doses produce faster growth. If UK licensing is upheld, poorer height velocities may result.
2. There was little data on the effect of HGH on the Quality of Life. There is a national multi-centre study (supported by the British Society for Paediatric Endocrinology and Diabetes) currently in progress in the UK which should add to this evidence base considerably. It seems rather odd that this appraisal consultation document has been drafted before the results of this study are known.

Are the summaries of clinical and cost effectiveness reasonable interpretations of the evidence?

The College thinks that the estimated cost of HGH treatment for a height gain of 1cm for each of the licensed indications for HGH treatment is reasonable. However, we think that the data used to estimate the cost of quality-adjusted life years (QALYs) was very limited in its scope and gave very little consideration of the effect of HGH treatment on Quality of Life, both in childhood and adulthood, and on the effect of an increased height velocity in childhood. We believe the utility values used in the manufacturer's and assessment group's economic models are likely to underestimate both the true disability associated with growth failure and the utility gained from HGH treatment.

Are the provisional recommendations sound and a suitable basis for guidance to the NHS?

The College thinks that the provisional recommendations of the Appraisal Committee seem generally sound. Hopefully, the findings will give further support to the cost-effectiveness of HGH treatment in its current clinical use.

The College is concerned about the advice to use the HGH products with the lowest acquisition costs where possible. Anecdotal evidence shows that allowing patients to choose their preferred HGH device improves adherence. Although most HGH products are biochemically identical, different injection devices and home care packages result in important distinctions between products. We note that biosimilar products such as Omnitrope have not had widespread clinical usage. We recommend that these cheaper products may be offered to patients but not encouraged over their more conventional expensive counterparts.

Are there any aspects of the recommendations that need particular consideration to ensure we avoid unlawful discrimination against any group of people on the grounds of gender, race, disability, age, sexual orientation, religion or belief?

The College notes that individuals with Prader Willi syndrome have learning difficulties and the indications for the use of HGH treatment in this condition are more complex than for height gain alone, with more subtle outcomes. The evidence base for the effectiveness of HGH treatment in this condition is rather thin. It is important that careful consideration is given to any recommendations made for this group in view of these complexities.

If you have any questions, please contact the RCPCH Clinical Standards team by email on clinical.standards@rcpch.ac.uk or by telephone on 020 7092 6175.

Kind regards



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