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Title **Clinical Effectiveness and Cost-effectiveness of Growth Hormone in Children: A Systematic Review and Economic Evaluation**

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Aim

This review considers the clinical effectiveness and cost effectiveness of growth hormone (GH) therapy in children with growth hormone deficiency, Turner syndrome, chronic renal failure, Prader-Willi syndrome, or idiopathic short stature.

Conclusions and results

The assessment of clinical effectiveness included 34 publications reporting 32 studies. Short-term growth and final height outcomes, and also body composition and psychological outcomes were evaluated. Jadad quality scores ranged from 1/5 to 4/5. Economic evaluations were not found, nor were studies reporting appropriate quality-of-life measures.

Although the quality of evidence varied, the studies suggest that GH treatment can increase short-term growth and improve final height. The reported effects of GH on short-term growth should be considered more reliable due to higher quality evidence. Effects of GH on final height should be considered with caution since the quality of studies is poorer. Results suggest that the effects of GH on short-term growth velocity (at 1 year) can range from no improvement to approximately 1 standard deviation above the normal growth velocity for children of the same age. Final height gains for treated children over untreated children range from about 2cm to 11cm.

GH treatment is expensive. The lifetime incremental cost of treating one child with GH ranges from £43,100–53,400 (for GHD) to £55,500–83,000 (for PWS). These costs, applied to children aged 8–15 years with the analyzed indications in England and Wales, yield total discounted costs of £904 million for complete treatment. The cost to treat children only in the four licensed conditions would be about £180 million. The data suggest that, under base case conditions, the incremental cost per centimeter gained in final height is approximately £6000 for GHD, £16,000–17,400 for TS, £7400–24,100 for CRF, £13,500–27,200 for ISS, and possibly around £7030 for PWS. Impacts of parameter values were evaluated by sensitivity analyses. The economic evaluation is limited by the quality of the trials.

Recommendations

The evidence suggests that prescribing GH to children improves their short-term growth and/or their final height (although the quality of evidence varies). GH treatment is expensive. Since a minority of children with licensed conditions currently receive GH, the budgetary impact of large increases in prescribing would be substantial.

Methods

Main electronic databases were searched (English language) up to April 2001. Bibliographies of related papers were assessed for relevant studies, and experts were contacted for advice, peer review, and to identify other references. Manufacturer submissions to the National Institute for Clinical Excellence were reviewed. Studies that met specified criteria were included, data were extracted, and quality was assessed. RCT quality was assessed using Jadad criteria, and non-RCTs were assessed using modified Spitzer criteria. Internal validity of economic evaluations was assessed using the BMJ checklist , and external validity was assessed using a series of relevant questions. Clinical effectiveness of GH in children was synthesized via a narrative review, with full tabulation of study results. A cost-effectiveness model was constructed using the best available evidence and applied to a UK setting.

Further research/reviews required

Large, multicenter RCTs are needed. These RCTs should focus on final height, which is the best outcome for assessing the effectiveness of GH, and should address quality-of-life factors for use in economic modeling.

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