Economic implications of growth hormone use in patients with short bowel syndrome

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Record Status
This is a critical abstract of an economic evaluation that meets the criteria for inclusion on NHS EED. Each abstract contains a brief summary of the methods, the results and conclusions followed by a detailed critical assessment on the reliability of the study and the conclusions drawn.

Health technology
The study examined the use of somatropin (rDNA origin) compared with parenteral nutrition (PN) alone for the treatment of patients with short bowel syndrome (SBS).

Type of intervention
Treatment.

Economic study type
Cost-effectiveness analysis.

Study population
The hypothetical study population comprised patients with SBS matching the inclusion criteria of the original somatropin trial (Byrne et al. 2005, see ‘Other Publications of Related Interest’ below for bibliographic details).

Setting
The setting was unclear, although it was likely to be secondary or tertiary. The economic analysis was conducted in the USA.

Dates to which data relate
The clinical data were collected from literature published between 1983 and 2005, with the main effect parameter taken from a 2005 study. Resource use was derived from published source between 1998 and 2004. The price year was 2004.

Source of effectiveness data
The clinical parameters associated with the model included:

the effectiveness of somatropin on PN;

the annual event rates for biliary problems, metabolic bone disease, end-stage renal disease, end-stage liver disease and death; and

the adverse event rates for peripheral oedema, facial oedema, arthralgia, myalgia and carpal tunnel syndrome.

Somatropin (rDNA origin) was assumed to have no effect on disease-related complications or survival, except for a 50.0% decrease in the risk of end-stage liver disease

Modelling
A discrete event simulation model was used to examine the costs and benefits. This model enabled the simulation of individual patient pathways. The time horizon of the model was 2 years. Patients were removed from the simulation
upon death or at the end of the time horizon. A graphical illustration of the model structure was provided.

**Sources searched to identify primary studies**
The main clinical effect parameter was derived from an 18-week Phase III pivotal clinical trial. There were no details of the study designs from which the other clinical parameters were obtained.

**Methods used to judge relevance and validity, and for extracting data**
The process used to identify the data was not reported. No inclusion criteria for any parameters were specified. The method used to select the estimates was neither reported nor discussed.

**Measure of benefits used in the economic analysis**
The authors did not derive a summary measure of benefit. This study was, in effect, a cost-consequences analysis.

**Direct costs**
The direct costs were those to the health care payer. These included the costs of hospitalisation, outpatient management, drugs or solutions, and laboratory tests. The resource use data were obtained from a published source. The cost of the drugs came from the drug manufacturer. A cost-to-charge ratio (0.61) was applied in order to produce a better reflection of the true costs. The resource quantities and the unit costs were reported separately. Discounting was not carried out. The price year was 2004.

**Statistical analysis of costs**
The costs were treated deterministically.

**Indirect Costs**
In line with the perspective adopted, the productivity costs were not included.

**Currency**
US dollars ($).

**Sensitivity analysis**
One-way sensitivity analyses were carried out to investigate the effects of variation in key inputs, including discount rate, cost of somatropin (rDNA origin), and other cost inputs. A multivariate sensitivity analysis was conducted, taking variability in treatment effect and costs into account, to estimate the range of possible outcomes.

**Estimated benefits used in the economic analysis**
Ninety-six per cent of the patients receiving somatropin reduced or eliminated PN within 6 weeks. Amongst these patients, 35.3% required no PN. The average use of PN was reduced by 2.8 days.

Patients receiving somatropin (rDNA origin) were estimated to suffer 2.9% fewer occluded catheters, 2.1% fewer superficial infections, 0.3% fewer tunnel infections and 6.0% fewer occurrences of sepsis.

**Cost results**
The costs for patients receiving PN were estimated to be $118,098 in year 1 and $132,935 in year 2.

With somatropin, the costs dropped to $84,309 in year 1 and $81,250 in year 2.
Over 2 years the cost-savings totalled $85,474.

Synthesis of costs and benefits
The costs and benefits were not combined.

Authors' conclusions
Somatropin use reduced the need for parenteral nutrition (PN) and resulted in cost-savings in health care.

CRD COMMENTARY - Selection of comparators
The rationale for choosing PN alone as the comparator was clear. It represented standard practice in the authors' setting. You should decide if it represents a valid comparator in your own setting.

Validity of estimate of measure of effectiveness
The main parameter was derived from a published clinical study, some details of which were reported. However, the sources of other clinical parameters were not described. In addition, the authors did not report search methods or inclusion criteria, nor did they provide a justification for their selection of the estimates. It was unclear whether the methods used to synthesise the evidence and to estimate the effectiveness of combination therapy were appropriate, as they were not described in full.

Validity of estimate of measure of benefit
The authors did not derive a summary measure of benefit, hence a cost-consequences analysis was performed. This was acceptable given that the full somatropin (rDNA) strategy was dominant. The study did not include quality of life measurements.

Validity of estimate of costs
The analysis was performed from the perspective of a health care payer. As such, all the relevant categories of cost appear to have been included in the study. When necessary, a cost-to-charge ratio was applied in order to reflect the true cost. Discounting would have been appropriate given the 2-year time horizon, but was not applied in the base-case. This was tested in sensitivity analysis with three rates being compared (1.5, 5 and 10%). The price year was reported and the costs and the quantities were reported separately; these will aid generalisability. Uncertainty in the total costs of events was evaluated in sensitivity analyses.

Other issues
The authors compared their findings with those from other studies and stated that their results were consistent with published figures. The authors acknowledged some limitations to their study. First, the effectiveness evidence was derived from a single, relatively short-time clinical trial. Second, because of insufficient data, assumptions had to be made for some model inputs. Third, the authors recognised that discrete event simulation might be perceived as a limitation because it was a new technique in the field of pharmacoeconomics. The results of the sensitivity analysis suggested that the results were robust to variations in key parameters.

Implications of the study
The authors suggested additional studies to determine the effect of treatment in populations other than the one studied. Overall, they concluded “somatropin use improves quality of life by reducing the need for parenteral nutrition and results in health care cost savings”.

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Other publications of related interest
Because readers are likely to encounter and assess individual publications, NHS EED abstracts reflect the original publication as it is written, as a stand-alone paper. Where NHS EED abstractors are able to identify positively that a publication is significantly linked to or informed by other publications, these will be referenced in the text of the abstract and their bibliographic details recorded here for information.


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MeSH
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