Intrasphincteric botulinum toxin versus pneumatic dilatation for achalasia: a cost minimization analysis

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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 compares pneumatic dilation (PD) to intrasphincteric botulinum toxin injection (IBTI) in the provision of symptom relief for patients with achalasia.

Type of intervention
Treatment.

Economic study type
Cost-effectiveness analysis.

Study population
The study population comprised people with achalasia. The study sample was divided into two different patient groups, one receiving PD and the other IBTI. The PD population comprised 114 achalasia patients treated between March 1986 and May 1987 by one of the authors. Follow-up data was available for 75 of the 114 patients treated by PD. The average age of patients in the PD population was 43 +/-18 (range: 12 - 78), and the male/female gender ratio was 44:31. The IBTI population comprised 31 patients included in one published study, the median age of patients was 55 (range: 19 - 85), and the gender ratio was 13:18.

Setting
The setting was secondary care. The economic study was carried out in Ontario, Canada.

Dates to which data relate
The probability and resource use data were collected between 1986 and 1997. The price year was not reported.

Source of effectiveness data
The effectiveness data were taken from a review/synthesis of completed studies supplemented by data from one hospital.

Modelling
The authors used a decision-analytic model to compare the direct costs of PD and IBTI for the treatment of achalasia.

Outcomes assessed in the review
The outcomes used as input parameters to the model were as follows:

the probability of response after initial treatment;
the probability of relapse requiring re-treatment;
the probability of requiring additional maintenance treatments;
the probability of a successful outcome with maintenance treatments; and
the probability of perforation with PD.

Study designs and other criteria for inclusion in the review
The authors did not report the design of the published studies included in the review. The unpublished data used were from a review of case notes supplemented by information from telephone follow-up with patients treated by PD. Patients included in the case note review had a confirmed diagnosis of achalasia. The authors did not report any other inclusion or exclusion criteria.

Sources searched to identify primary studies
The authors did not report the sources searched to identify primary studies.

Criteria used to ensure the validity of primary studies
The authors did not report whether any criteria were used to ensure the validity of primary data sources.

Methods used to judge relevance and validity, and for extracting data
The authors did not report the use of criteria to judge the relevance and validity of data or methods used to extract data.

Number of primary studies included
The authors referenced 4 published and 1 unpublished primary data sources used to provide probability estimates.

Methods of combining primary studies
The primary data sources were not combined to generate individual probability estimates for this model.

Investigation of differences between primary studies
The authors did not report any differences between primary published data sources, in terms of participants, intervention or outcome measures. The authors reported the patient characteristics of 1 published study and the unpublished data used, but did not report whether any formal comparison was undertaken.

Results of the review
The results for the outcomes used as input parameters to the model were as follows:

the probability of response after initial treatment: PD = 0.91, IBTI = 0.65;
the probability of relapse requiring re-treatment: PD = 0.61, IBTI = 0.95;
the probability of requiring additional maintenance treatments: PD = 0.40, IBTI = 0.95;
the probability of a successful outcome with maintenance treatments: PD = 0.64, IBTI = 0.53; and
the probability of perforation with PD = 0.04.
Measure of benefits used in the economic analysis
No summary measure of health benefit was used in the economic analysis. The outcomes were reported in a
disaggregated way and, as such, this was a cost-consequences study.

Direct costs
The authors reported some resource use in addition to the following direct costs used as inputs to the analysis:

the cost of each treatment session: PD = $829, IBTI = $662;

the cost of surgical thoracotomy and myotomy: PD = $7,900, IBTI = $7,900; and

the cost of surgical thoracotomy and myotomy with complications: PD = $20,050, IBTI = $2,050.

The authors reported that summing inpatient and outpatient hospital costs, including supplies, nursing costs, and
physician fees, derived treatment costs. However, costs involved in the initial diagnosis were not included. The
information was obtained from a combination of the Ontario Schedules of fees and hospital administration records.
The cost of a PD session included the cost of fluoroscopy, an overnight inpatient stay, and a post-procedure
radiological contrast study. IBTI session costs were equivalent to an outpatient gastroscopy with the additional
expense of 100 units of botulinum toxin A. The authors reported that the cost of surgical myotomy was arrived at
similarly and included surgical and anaesthesia fees, operating costs, nursing, 1 day in an intensive care setting, and 7
days of ward care. Surgical complications incurred an additional cost of 5 days of intensive care followed by 14 days
of ward care. Costs were discounted at a rate of 5% per annum. The price year was not reported.

Statistical analysis of costs
No statistical analysis was reported.

Indirect Costs
No indirect costs were included in the analysis.

Currency
Canadian dollars (Can$). A currency conversion of Can$1.00 = US$0.65 was reported.

Sensitivity analysis
The authors performed one-way sensitivity analysis on costs and probabilities used in the model in order to assess the
impact on the results.

Estimated benefits used in the economic analysis
See effectiveness/probability data above.

Cost results
The authors reported that the total estimated cost of the PD treatment strategy was Can$3,608, and that the total
estimated cost of the IBTI treatment strategy was Can$5,033.

Synthesis of costs and benefits
No synthesis of costs and benefits was carried out and no incremental analysis was reported. The authors reported that
the sensitivity analyses confirmed that PD is a more economical approach for the management of achalasia: even over
a wide range of cost and probability scenarios. The authors reported threshold values at which the total cost of
treatment would be equivalent. However, they also reported that most of the thresholds did not fall within ranges that were clinically relevant or realistic. For example the authors reported that the two treatment strategies would have equivalent costs if the risk of perforation with PD rose to 54% or the probability of acquiring IBTI fell to 51%. The authors did, however, state that the cost of the IBTI strategy and the PD strategy is equivalent if a patient's life expectancy is 2 years, and that the cost of the IBTI treatment strategy is lower than the PD strategy when the patient's life expectancy is below 2 years.

**Authors' conclusions**

The authors concluded that IBTI is more costly than PD for the treatment of achalasia and that the added expense of frequent re-treatment with IBTI outweighs the potential economic benefits of the safety of the procedure, unless life expectancy is 2 years or less.

**CRD COMMENTARY - Selection of comparators**

A justification was given for the choice of comparator used, namely that it represented the lower cost alternative of 2 interventions (PD or surgical myotomy) that were current practice in the authors setting. You, as a user of the database should decide if this is a widely used and appropriate health technology in your own setting.

**Validity of estimate of measure of effectiveness**

The authors did not report whether a systematic review of the literature had been undertaken. The impact of the differences between primary studies when estimating probabilities was not considered. The authors used data from a review of case notes of people treated by PD over the previous 10 years, but did not report whether there were changes in the way PD treatment was given or whether there were other factors over this time period which could have affected the values of the probability data generated. The authors also report that there was a 30% loss to follow-up. There was no direct comparison of the long-term effectiveness of the alternatives, by generating a measure of outcome from the model (e.g. life year gained, percentage of people cured or gaining symptom relief). The evaluation therefore cannot identify which alternative is most effective in terms of long term patient health. However, the authors did acknowledge that there were differences in the long term safety and effectiveness of the 2 interventions. The authors did report a one-way sensitivity analysis of all the probability data used and this indicated that the results were robust to changes in all but 1 of the parameters (life expectancy of the patient). The authors also reported that they biased the analysis in favour of IBTI (the higher cost alternative).

**Validity of estimate of measure of benefit**

The authors did not derive a summary measure of health benefit and the analysis should therefore be categorised as a cost-consequences study. The authors reported a cost-minimisation analysis but did not demonstrate clinical equivalence.

**Validity of estimate of costs**

All categories of cost relevant to the perspective adopted were included in the analysis. Some relevant costs were, however, omitted from the analysis. The authors did not include the costs involved in the initial diagnosis and the potential long-term costs of surgical myotomy were not included as these were stated to have occurred beyond the 10-year interval. Costs and quantities were not consistently reported separately. No statistical analysis of costs or probabilities was performed. However, the authors did conduct a sensitivity analysis of costs and probabilities, and the ranges specified appeared appropriate. Costs were discounted at a standard rate of 5% per annum. The authors performed appropriate currency conversions, but did not report the price year.

**Other issues**

The authors made appropriate comparisons of their findings with those from other studies and the issue of generalisability to other settings was addressed. The authors report that their study is limited by the fact that the validity of the modelling approach to cost analysis depends on the reliability of the input variables. However, they
stated that the study by Pasricha et al (1995) provides a comprehensive report of the long-term outcome after IBTI for achalasia, and the authors reported that they used this study to directly obtain and estimate the input parameters used.

**Implications of the study**
The authors conclude that IBTI is more costly than PD for the treatment of achalasia and that the added expense of frequent re-treatment with IBTI outweighs the potential economic benefits of the safety of the procedure, unless life expectancy is 2 years or less. The authors report that their analysis does not consider clinical effectiveness or quality-of-life, and state that this must await the results of trials directly comparing the results of these two treatments. However, the authors do argue that based on IBTI experience thus far, it is unlikely that IBTI will provide any important clinical advantage to negate the cost benefit of PD as the treatment of choice for achalasia.

**Source of funding**
Supported by a Medical Research Council of Canada grant MT-12608.

**Bibliographic details**

**PubMedID**
10502169

**Other publications of related interest**

**Indexing Status**
Subject indexing assigned by NLM

**MeSH**
Adolescent; Adult; Aged; Botulinum Toxins /administration & dosage /economics; Canada; Catheterization /economics; Child; Cost Savings; Cost-Benefit Analysis; Costs and Cost Analysis; Decision Support Techniques; Esophageal Achalasia /economics /therapy; Esophagogastric Junction; Female; Humans; Injections; Male; Middle Aged

**AccessionNumber**
21999001853

**Date bibliographic record published**
30/04/2002

**Date abstract record published**
30/04/2002