Pneumatic dilation versus intrasphincteric botulinum toxin injection in the treatment of achalasia cardia in India: an economic 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
Pneumatic dilation (PD) with a Rigiflex balloon of 35 mm diameter was compared with intrasphincteric botulinum toxin (BT) injection (Botox; Allergen Irvin, CA, USA). For the intrasphincteric injection, 20 units were injected into each of the four quadrants of the lower oesophageal sphincter with a sclerotherapy needle.

Type of intervention
Treatment.

Economic study type
Cost-effectiveness analysis.

Study population
The hypothetical study population comprised patients with achalasia cardia. A typical patient was reported to be older than 40 years and to not have megaesophagus or oesophageal cancer. The presence of the latter two co-morbidities would have indicated that surgery was the preferred treatment option.

Setting
Secondary care.

Dates to which data relate
The effectiveness data were taken from papers published between 1995 and 2001. The price year was not stated.

Source of effectiveness data
The effectiveness data were derived from a review and synthesis of completed studies, augmented with the authors’ assumptions.

Modelling
A decision tree model (Data 4.0; TreeAge Software, USA) was used to compare the two treatments. The time horizon used was "only up to one recurrence after successful PD and two recurrences after successful BT".

Outcomes assessed in the review
The authors assessed the following outcomes in their review as probabilities:
the response to PD;
the response to BT;
perforation after PD;
the need for emergency surgery following perforation with PD;
the need for repeat PD after successful PD, due to recurring symptoms;
the need for repeat BT after successful treatment with initial BT;
the response to re-treatment with BT; and
the frequency of complications after emergency surgery.

**Study designs and other criteria for inclusion in the review**
The authors did not report that a systematic review had been conducted.

**Sources searched to identify primary studies**
MEDLINE was searched for publications in English.

**Criteria used to ensure the validity of primary studies**
Not reported.

**Methods used to judge relevance and validity, and for extracting data**
The authors stated that probability data from Indian studies were preferred where available. No other methods to assess relevance or validity were reported.

**Number of primary studies included**
Sixteen primary studies were used in the review.

**Methods of combining primary studies**
Not reported.

**Investigation of differences between primary studies**
Not reported.

**Results of the review**
The baseline probabilities found from the review are presented here along with the sensitivity analysis range (in brackets):

- the response to PD was 81 (70 - 93) and the response to BT was 80 (70 - 90);
- perforation after PD was 4 (2 - 15);
- the need for emergency surgery following perforation with PD was 50 (20 - 100);
- the need for repeat PD after successful PD, due to recurring symptoms, was 20 (10 - 30);
the need for repeat BT after successful treatment with initial BT was 90 (70 - 98);

the response to re-treatment with BT was 90 (85 - 95); and

the frequency of complications after emergency surgery was 19 (0 - 54).

Methods used to derive estimates of effectiveness
The authors made assumptions to augment the effectiveness results.

Estimates of effectiveness and key assumptions
The three assumptions were:

all patients with post-PD perforation needed myotomy after closure of perforation on conservative management;

myotomy was successful in all cases where treatment with PD or BT had failed; and

there were no complications following elective myotomy despite prior treatment with PD or BT.

The authors reported that the last assumption was made despite some studies reporting evidence to the contrary.

Measure of benefits used in the economic analysis
The measure of benefit in the economic analysis was the number of patients treated successfully.

Direct costs
A patient perspective was adopted for the cost analysis. The authors did not report discounting, which seems appropriate as the time horizon appears to have been within 2 years. The costs were reported to have been estimated from the authors' institution and several private and government institutions in India. The unit costs were estimated and reported separately for PD (per session), BT injection (per session), elective myotomy, conservative treatment for perforation, emergency surgery for complications following dilation, and the treatment of complications following emergency surgery. The quantities were not explicitly reported, but appear to have been defined by the treatment pathway taken by the patient and described by the decision tree model. No price year for the analysis was stated.

Statistical analysis of costs
Not reported.

Indirect Costs
The authors stated that only the direct costs were considered. If pneumatic dilation required a stay in hospital, then the cost of the patient’s time may have been relevant to this study.

Currency
Indian rupees (R). For reference, the authors reported that the current exchange rate was US$1.00 = R49.00. The year was not specified.

Sensitivity analysis
Sensitivity analyses were conducted on both the probability and cost parameters used to populate the model. It was not reported how the specific ranges used for the costs estimates were defined. One-way and two-way sensitivity analyses were carried out.
Estimated benefits used in the economic analysis
Neither the absolute nor the incremental benefits were reported.

Cost results
The cost of BT was R18,520 per patient, while the cost of PD was R4,568 rupees per patient.

Synthesis of costs and benefits
The incremental cost of BT per successfully treated patient was R13,952.

The authors reported that the frequency of oesophageal perforation following PD did not alter the conclusions from the baseline analysis. They also reported that, in the two-way sensitivity analysis, BT remained the most costly treatment. They found that when the cost of BT was below a certain threshold, and the cost of PD was above a certain threshold, BT was the cheaper alternative.

Authors' conclusions
Botulinum toxin (BT) was a costlier strategy than PD for treating achalasia cardia in India. The authors did not draw any conclusions about effectiveness as a result of their study.

CRD COMMENTARY - Selection of comparators
The authors compared BT treatment with PD treatment. They justified PD as a comparator with reference to the fact that PD represented an established treatment option.

Validity of estimate of measure of effectiveness
The authors did not state that a systematic review of the literature was undertaken. They did not discuss how the published effectiveness estimates were combined to derive the baseline parameter values. They also did not discuss differences between the estimates reported in the primary studies, or the potential impact of these differences when deriving their baseline values.

The authors supplemented their effectiveness estimates with some assumptions. These were not well justified, and in one case the authors suggested that evidence existed to the contrary.

Validity of estimate of measure of benefit
The authors estimated the cost per successfully treated patient, but did not report the number of successfully treated patients for each treatment regime. This makes it difficult for the reader to form a complete understanding of the research results. The number of successfully treated patients was modelled using a decision tree format, which was appropriate.

Validity of estimate of costs
The authors stated that their study was carried out from the perspective of the patient. It was not clear from the discussion whether the patient had to pay directly for all of the cost elements included. The costs included seem to have been appropriate for a hospital perspective. In addition, the authors did not include the patients' potential travel costs and time lost from work. These omissions may not have affected the principle conclusions due to the substantial difference in costs between the treatment strategies. The unit costs were reported separately but the quantities were not.

Other issues
The authors made appropriate comparisons of their findings with those from other published results and discussed the reasons for the differences between them. The authors did not discuss the generalisability of their results to other
settings. They did, however, state that their sensitivity analysis “indicates the robustness of our analysis, and the applicability of our results to both 'for profit' private hospital and 'non-profit' government institutions in India”. This is not necessarily the case. The authors carried out their analysis from the perspective of the patient. An analysis from alternative perspectives would require the inclusion of potentially different cost elements and may substantially alter the results achieved. The authors presented their results selectively in the sense that they did not report the cost and effectiveness aspects separately.

The authors appropriately presented some limitations of their study. These included a bias towards BT and the relatively short time horizon.

**Implications of the study**
The authors made no explicit recommendations for changes in policy or practice following their study, but it is clear that they do not favour BT as a first-line treatment option. The authors appropriately stated that their conclusions do not hold for patients with megaesophagus, and that the optimum treatment for these patients requires further investigation.

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None stated.

**Bibliographic details**

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**Other publications of related interest**

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