A cost-utility analysis of therapy for amblyopia

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 treatment of paediatric amblyopia was examined. Several medical and surgical treatments, according to the American Academy of Ophthalmology Preferred Practice Pattern for Amblyopia, were considered. It was assumed that all cases of amblyopia were treated medically and 80% of cases did not require surgical intervention. However, 17% of cases required ocular alignment, 1.5% cataract extraction and 1.5% frontalis suspension. Treatments were performed for several types of amblyopia, primarily strabismic, anisometropic or refractive, and deprivation.

Type of intervention
Treatment.

Economic study type
Cost-utility analysis.

Study population
The study population comprised a hypothetical cohort of children aged 4 years and suffering from amblyopia.

Setting
The setting was a paediatric ophthalmology practice. The economic study was carried out in the USA.

Dates to which data relate
The effectiveness and resource use data were obtained from studies published between 1980 and 2000. The price year was 2001.

Source of effectiveness data
The effectiveness evidence was derived from published studies and the authors' assumptions.

Modelling
A decision tree model was constructed to simulate the clinical and economic outcomes associated with treatment, or no treatment, in a hypothetical cohort of children with amblyopia. The mortality data were combined with treatment results expressed as utility values, in order to calculate the quality-adjusted life-years (QALYs). A simplified version of the decision tree was reported graphically.

Outcomes assessed in the review
The outcomes estimated in the review were:

the life expectancy for the reference-case patient (aged 4 years);
visual acuity results before and after treatment;
changes in visual acuity with age;
the proportion of accommodative strabismic amblyopia;
the sub-group of strabismus patients with amblyopia who would undergo alignment surgery of all cases of strabismic amblyopia;
the failure rate associated with lyophilised fascia lata; and
the recurrence rate with supramid.

Study designs and other criteria for inclusion in the review
The life expectancy was obtained from the Centers for Disease Control and Prevention/National Center for Health Statistics. The design of the remaining primary studies was not reported.

Sources searched to identify primary studies
Not stated.

Criteria used to ensure the validity of primary studies
Not stated.

Methods used to judge relevance and validity, and for extracting data
Not stated.

Number of primary studies included
The effectiveness data used in the decision model were derived from approximately 17 primary studies.

Methods of combining primary studies
The methods used to combine the studies were not reported.

Investigation of differences between primary studies
Not stated.

Results of the review
The life expectancy for the reference-case patient was 77 years.

The mean visual acuity was 20/80 before treatment and 20/32 after treatment.

In terms of the changes in visual acuity with age, among patients aged 43 to 86 years, 2.9% had visual acuity reduced to less than 20/40, and 0.3% had vision reduced to less than 20/200 in the better eye. Patients older than 75 years were 12.5 times more likely to have vision reduced to less than 20/40, and were 78 times more likely to have vision reduced to less than 20/200 in the better eye.

The proportion of accommodative strabismic amblyopia ranged from 60 to 75%. Sixty per cent of accommodative estropia cases were not fully correctable with spectacles, and thus required surgery.
The sub-group of strabismus patients with amblyopia who would undergo alignment surgery ranged from 48 to 62% of all cases of strabismic amblyopia.

The failure rate associated with lyophilised fascia lata was 6 to 8% within 18 months, 10% for 2 to 3 years after surgery, and 50% for 8 to 9 years after surgery.

The recurrence rate with supramid was up to 100% of patients within 18 months.

**Methods used to derive estimates of effectiveness**
The authors made some assumptions, partly supported by the literature, which were used in the decision model.

**Estimates of effectiveness and key assumptions**
It was assumed that:

- amblyopia did not affect life expectancy;
- the contralateral eye had 20/20 vision after treatment;
- the mean utility value corresponding to vision of 20/20 in one eye and 20/80 in the second eye (untreated amblyopia) was 0.83 (using a database of 35 consecutive patients);
- the mean utility value for 40 patients with 20/20 in one eye and 20/32 in the second eye (treated amblyopia) was 0.86, thus the treatment led to a gain of 0.03 utility units per year;
- about 17% of amblyopes underwent alignment surgery.

Further assumptions were also made.

**Measure of benefits used in the economic analysis**
QALYs were used as the benefit measure in the economic analysis. They were obtained by combining data on survival and utility values, which were calculated using the time-trade-off approach. A 3% annual discount rate was used.

**Direct costs**
A 3% annual discount rate was applied since lifetime costs were evaluated. The unit costs and the resources used were not analysed separately. The health services included in the economic analysis were physician visits, drugs, optical-wear services and surgical interventions. Nonsurgical expenses included spectacles, 3M Occluder, atropine, and prednisolone and tobramycin. The cost/resource boundary adopted in the study was that of the third-party payer. The authors stated that high-ended values were used to represent maximum cost values. The costs were estimated using the reimbursement rates paid by the Health Care Financing Agency according to Current Procedural Terminology (CPT) data. The drug costs were derived from average wholesale prices. The resource use data were derived from the authors’ assumptions and published studies. The price year was 2001.

**Statistical analysis of costs**
The costs were treated deterministically in the base-case.

**Indirect Costs**
The indirect costs were not included in the economic analysis.

**Currency**
Sensitivity analysis
One-way sensitivity analyses were conducted to evaluate the robustness of the estimated cost per QALY to variations in the discount rate (0 to 10%), estimated costs (+/- 10%) and estimated QALYs (+/- 10%).

Estimated benefits used in the economic analysis
The undiscounted QALYs gained with treatment relative to no treatment were 2.19, while the discounted QALYs were 0.7858. Adding 0.01475 discounted QALYs gained because of amblyopia treatment when the vision in the non-amblyopic eye is decreased, yielded 0.80055 QALYs gained from treatment in comparison with no treatment.

Cost results
The total estimated costs were not reported.

Synthesis of costs and benefits
An incremental cost-utility analysis was carried out to combine the costs and benefits of the interventions under study. The cost per QALY gained with amblyopia treatment relative to no treatment was $1,726 when only medical treatments were considered, and $2,281 when surgical treatments were included in the analysis. In the sensitivity analysis, the cost per QALY gained ranged from $637 (the most favourable scenario) to $4,973 (the most pessimistic scenario).

Authors' conclusions
Amblyopia treatment was highly cost-effective when compared with no treatment in the USA. Its cost per QALY gained was far below the commonly used threshold of $50,000 per QALY. In addition, it compared favourably with other ophthalmologic interventions funded in the health care system.

CRD COMMENTARY - Selection of comparators
The rationale for the choice of the comparator was clear. No treatment was selected since the aim of the study was to assess the additional value of treatment among children with amblyopia. The authors did not consider a single treatment, but a mix of medical and surgical treatments widely used to treat amblyopia, according to the recommendations of the American Academy of Ophthalmology. You should decide whether it represents a valid comparator in your own setting.

Validity of estimate of measure of effectiveness
The effectiveness data were mainly derived from published studies and the authors’ assumptions. Both sources of data were fairly mixed up in the paper. It appears that the authors made some assumptions that were supported by the literature, but it was unclear how such data were extracted from the published studies. A formal review of the literature was not undertaken and the designs of the primary studies were not reported. The data from the primary studies were combined, although the methods used were not described. It was not stated whether the primary studies were comparable in terms of the patients and interventions studied. Finally, the validity of such studies was not mentioned.

In terms of the assumptions made, the authors did not investigate their potential variability in the sensitivity analysis. These issues tend to cast some doubts on the internal validity of the effectiveness estimates used in the decision analysis.

Validity of estimate of measure of benefit
QALYs were used as the summary benefit measure in the economic analysis. This appears to have been appropriate, as it captures all mortality and morbidity aspects of the interventions from the perspective of the patients. Indeed, patient-
based utility values from a published study, which used the time-trade-off method, were used. Details of the decision model used in the economic analysis were reported and the model was shown graphically in the paper. The use of QALYs enhances the comparability of the benefits of the present study with those obtained with other interventions implemented in the health care system. The number of QALYs gained was varied in the sensitivity analysis.

Validity of estimate of costs
The perspective adopted in the study was stated. It appears that all the relevant categories of costs have, appropriately, been included in the analysis. The authors stated that amblyopia screening costs and societal costs (school absences, iatrogenic psychosocial effects of occlusion therapy) were not included in the analysis. However, their potential inclusion would lead to the conclusion that amblyopia treatments may contribute substantially to the earning power of an affected individual and the Gross Domestic Product in the USA.

A detailed breakdown of the costs was provided and the unit costs were reported according to CPT codes, thus making the costs used representative of costs across the USA. The price year was given, thus simplifying reflation exercises in other settings. However, details of resource use were not reported. Also, the authors made several assumptions, which do not appear to have been supported by the literature or expert opinion. The costs were treated deterministically and only the total costs were varied in the sensitivity analysis. The authors discounted only those categories of costs that were incurred in the long term, while those observed in the short term were not discounted. The source of cost data was reported. The authors commented that strabismus treatment costs should have been included in some cases, as some experts consider strabismus and amblyopia strictly related.

Other issues
The authors made several comparisons of their findings with those from other studies evaluating ophthalmologic interventions and other technologies. The issue of the generalisability of the study results to other settings was not addressed. Only limited sensitivity analyses were conducted, more often to evaluate the robustness of the study results than to estimate the variability in data. The study referred to a population of children with amblyopia and this was reflected in the conclusions of the analysis.

Implications of the study
The study results suggest that amblyopia treatment was cost-effective from the perspective of the third-party payer. This information is of particular importance for a public decision-maker as amblyopia represents a major public health issue. The authors estimated that, at a conservative prevalence rate of 2%, there would be about 5,600,000 people in the USA that would benefit from amblyopia treatment. However, caution is required when interpreting the conclusions of the study, due to the limitations of the analysis.

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