Different desferrioxamine usage in the patients with thalassemia major: a cost-effect 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
Two methods of administering desferrioxamine (DFO) to patients with thalassemia major (TM) were examined. One approach was the continuous subcutaneous infusion of DFO (50 mg/kg per day) for 48 hours with an infusor pump (total of 100 mg/kg DFO in 2 days). The other was the intermittent infusion of DFO (50 mg/kg per day) for 4 days per week with a classic pump.

Type of intervention
Treatment.

Economic study type
Cost-effectiveness analysis.

Study population
The study population comprised patients with TM. No specific inclusion or exclusion criteria were reported.

Setting
The authors did not specify the study setting. The economic study was carried out in Turkey.

Dates to which data relate
The dates during which the effectiveness and resource use data were collected were not reported. The price year was not reported.

Source of effectiveness data
The effectiveness data were derived from a single study.

Link between effectiveness and cost data
The same patient sample provided both the effectiveness data and the cost data. It was unclear whether the costing was carried out prospectively or retrospectively.

Study sample
Power calculations were not reported. The 54 patients with TM that were included in the analysis were divided into two groups. The first group comprised patients who were infused DFO 100 mg/kg in 2 days using an infusor pump (infusor group). The second group comprised patients who were infused DFO 200 mg/kg in 4 days using a classic pump (classic group). The infusor group contained 27 patients (18 female) aged between 5.5 and 20.5 years, while the classic group contained 27 patients (18 female) aged between 6 and 22 years.
Study design
This was a non-randomised controlled study that was conducted in a single centre. The follow-up was one year for both groups. No blinding of the outcome assessment was reported. No loss to follow-up was reported.

Analysis of effectiveness
It was unclear whether all the patients included in the sample were considered at analysis. The primary health outcomes assessed were the mean serum ferritin levels and compliance rates. The authors reported that compliance with DFO treatment is a major determinant of survival. They did not state whether the two groups were comparable at baseline in terms of demographic and clinical characteristics. Adjustments for confounding factors were not reported.

Effectiveness results
The mean serum ferritin levels before and after therapy for the infusor group versus the classic group were, respectively:

4,782.1 (+/- 2,254.6) versus 3,609.8 (+/- 2,079.3) before therapy;
3,316.6 (+/- 1,218.1) versus 2,869.5 (+/- 1,434.3) after 3 months;
3,830.9 (+/- 1,844.8) versus 3,447.6 (+/- 1,670.2) after 6 months; and
3,707.3 (+/- 1,670.8) versus 3,070.3 (+/- 1,442.9) after 12 months.

These differences were not statistically significant, (p>0.5).

Patient compliance was 100% in the infusor group and 80% in the classic group.

The authors reported that there were no meaningful distinctions between the two groups because of the number and essence of the complications (the results were not shown).

Clinical conclusions
The authors concluded that there was no statistical difference in iron loading between infusor and classic pumps. The findings indicated that the compliance rate was higher with the infusor pump than with the classic pump.

Measure of benefits used in the economic analysis
No summary measure of benefit was used. The study was, in effect, a cost-consequences analysis.

Direct costs
Discounting was not carried out as the costs were incurred during less than 2 years. The unit costs and the quantities were not analysed separately. Only therapy costs were included in the analysis. These costs were for outpatient visits, inpatient visits, blood transfusions, chelation and the treatment of complications. The unit costs were derived from the Ministry of Health. The resource use data appear to have been derived from patient records. The dates and the price year were not reported. The total and average costs per patient were reported.

Statistical analysis of costs
No statistical analysis of the costs was carried out.

Indirect Costs
The indirect costs were not included.
Currency
New Turkish lira (YTL). A conversion to US dollars ($) was also reported, although the rate used was not stated.

Sensitivity analysis
No sensitivity analysis was reported.

Estimated benefits used in the economic analysis
See the 'Effectiveness Results' section.

Cost results
The total cost was YTL 277,792.2 for the infusor group and YTL 377,369.8 for the classic group.

The cost per patient was YTL 10,251.6 ($7,070) for the infusor group and YTL 13,986.7 ($9,647) for the classic group.

Synthesis of costs and benefits
Not relevant as no summary benefit measure was derived.

Authors' conclusions
No statistical difference between the infusor pump and classic pump was found in terms of cost-effectiveness. However, from this study, it would appear that the compliance rate was higher and the costs were lower in the infusor pump group than in the classic pump group. Therefore, the infusor pump should be considered as a cost-effective alternative to the classic pump in patients with thalassemia major (TM).

CRD COMMENTARY - Selection of comparators
The authors explicitly justified their choice of the comparator. Continuous 48 hours' infusion with an infusor pump and intermittent 40 hours' infusion with a classic pump reflect two potential alternatives for DFO infusions in the authors' setting. However, continuous 72-hour, 96-hour or 120-hour DFO infusions with an infusor pump and intermittent 60 hours' DFO infusion with a classic pump were potential alternatives that were not considered in the analysis. You should judge whether these comparators are relevant in your own setting, or whether other comparators could have been relevant as well.

Validity of estimate of measure of effectiveness
The analysis was based on a non-randomised controlled study. The research design was not ideal in relation to the study question, owing to the inability to determine the comparability of the groups at baseline and to account for confounding factors potentially impacting on the results. In addition, the method of assigning patients to each group was unclear. The internal validity of the study was, therefore, considered to be poor. The lack of power calculations and the subsequent small sample size meant that it was unclear whether the study sample was sufficiently large to prove the statistically significant difference in health outcomes. The study sample appears to have been representative of the study population as there was no sample selection.

Validity of estimate of measure of benefit
The authors did not derive a measure of health benefits. The reader is referred to the comments in the 'Validity of estimate of measure of effectiveness' field (above).

Validity of estimate of costs
Although the perspective adopted in the analysis was unclear, it appears that those cost categories relevant to a healthcare perspective have been included. The authors provided limited details on the cost analysis. The costs and the quantities were not reported separately, which may hamper the extrapolation of this analysis to other settings. The resource use data were taken from the authors' setting, while the unit costs were taken from the Ministry of Health. The lack of any statistical or sensitivity analyses on both parameters potentially limits the interpretation of the findings. The failure to report the price year also limits any future reflation exercise. Discounting was, appropriately, not carried out since all the costs were incurred in less than one year.

Other issues
The authors compared their clinical results, but not the economic results, with those from other studies. The issue of generalisability to other settings was not addressed. The authors reported no limitations to their study. Although the authors do not appear to have presented their results selectively, the paper was limited by incomplete information (in both effectiveness and cost analyses) and by a misleading conclusion. For example, the complication rates were not reported even though the costs associated with complications were included in the analysis. The authors concluded that the infusor pump and classic pump were similar in terms of costs and effectiveness, but compliance in the infusor group was higher and the costs associated with the infusor were lower than in the classic pump group. Therefore, the infusor pump should be considered a cost-effective alternative to the classic pump in patients with TM.

Implications of the study
The authors made no specific recommendations for changes in policy or practice and/or the need for further research. An adequately powered, prospective, randomised controlled trial associated with an economic evaluation is needed to confirm the results of this study.

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None stated.

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