Cost-utility analysis of deferasirox compared to standard therapy with desferrioxamine for patients requiring iron chelation therapy in the United Kingdom

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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.

CRD summary
The aim was to compare the cost-effectiveness of deferasirox versus desferrioxamine, for the control of iron overload, in adults and children receiving frequent blood transfusions. The authors concluded that deferasirox was cost-effective due to the cost and quality of life benefits associated with a simpler and more convenient oral administration. The study appears to have been based on valid methodology, which enhances the validity of the authors’ conclusions.

Type of economic evaluation
Cost-utility analysis

Study objective
The aim was to compare the cost-effectiveness of deferasirox versus desferrioxamine (DFO) for the control of iron overload in patients (adults and children) receiving frequent blood transfusions, such as patients with β-thalassaemia. The analysis focused on the differences due to the mode of administration (oral versus intravenous) of the two treatments.

Interventions
The two strategies were deferasirox (20mg/kg per day or more) versus DFO (35mg/kg per day or more). DFO was administered as a slow subcutaneous infusion, usually over 8 to 12 hours a day, for five to seven days a week. Deferasirox was administered once a day by an oral tablet, dispersed in water or juice, to provide 24-hour treatment.

Location/setting
UK/tertiary care (hospital).

Methods
Analytical approach:
This economic evaluation was based on a decision analytic model with a one-year time horizon. The authors stated that the perspective of the British National Health Service (NHS) was adopted.

Effectiveness data:
The clinical data came from a selection of known, relevant studies, mainly clinical trials. The authors made a key assumption on the equal efficacy of the two treatments on the basis of data from a multicentre, open label, randomised controlled trial (RCT) of a sample of 586 children and adults. As there was no effectiveness or survival difference between the two treatments, the key clinical endpoint was the incidence of side effects related to treatment.

Monetary benefit and utility valuations:
The utility values associated with the mode of administration were derived from a community-based preference study involving a representative cross-section of 120 members of the UK general population and using the time trade-off technique. The disutility associated with treatment-related adverse events was derived from published studies.

Measure of benefit:
Quality-adjusted life-years (QALYs) were used as the summary benefit measure and they were estimated using the decision model, but were only driven by the health utilities, given the equal survival rate for the two treatments.
Cost data:
The health service costs were those of drugs (acquisition and administration), and treatment and monitoring of drug-related adverse events. The resource use and costs associated with equipment were based on a retrospective study involving 29 patients across four UK centres. The costs of treatment-related adverse events were based on published economic evaluations. Drug costs were derived from the British National Formulary and dosages came from the RCT that provided the clinical efficacy assumption. The costs were in UK pounds sterling (£) and the price year was 2007.

Analysis of uncertainty:
A multi-way sensitivity analysis was undertaken to test the impact of changes in the base-case assumptions about the patient weight, type of infusion device, and utility values. A probabilistic sensitivity analysis was also carried out by assigning probability distributions to the model inputs. The details of the types of distributions were reported.

Results
In the base-case scenario of a typical β-thalassaemia patient weighing 42kg, deferasirox reduced the total costs by £1,570 and led to a QALY improvement of 0.176 in comparison with DFO. Deferasirox was the dominant strategy because it was simultaneously less expensive and more effective. A similar conclusion was reached for a typical patient with sickle cell disease (SCD) weighing 52kg.

For a patient weighing 62kg, the incremental cost per QALY gained, with deferasirox over DFO, was £7,775 and for a 72kg patient it was £16,720.

The deterministic sensitivity analysis showed that, even when unfavourable inputs were used, the incremental cost per QALY gained with deferasirox over DFO was never more than £23,080. The probabilistic sensitivity analysis indicated that, at a willingness to pay of £20,000 per QALY, the probability of deferasirox being cost-effective was 80% and at £30,000 it was 85%.

Authors’ conclusions
The authors concluded that deferasirox was a cost-effective alternative to DFO, for iron chelation therapy, due to the cost and quality of life benefits associated with a simpler and more convenient oral administration.

CRD commentary
Interventions:
The selection of the comparators was appropriate in that DFO was the gold standard of care for chronic iron overload, while deferasirox was licensed in the UK in September 2006.

Effectiveness/benefits:
The selective approach used to identify the sources of data was intended to include the most relevant inputs known to the authors. Most of the clinical inputs came from RCTs, which are usually considered to be a robust source of data. Some details on the design and sample size of these studies were provided. The methodological approach used to derive the utility valuations was explicitly described and was based on a validated instrument. QALYs are an appropriate benefit measure, which permit cross-disease comparisons.

Costs:
The cost categories were consistent with the perspective. The unit costs and quantities of resources were presented separately for some cost categories such as the administration of DFO. The price year was reported, which enhances the possibility of making reflation exercises for other time periods Some sources of data were clearly reported, while other sources were not described.

Analysis and results:
The use of an incremental approach to synthesise the costs and benefits was appropriate and showed the dominance of one treatment over the other. The issue of uncertainty was appropriately investigated by means of both a deterministic and a probabilistic approach. Alternative scenarios were also considered. The authors pointed out that their findings referred mainly to patients with β-thalassaemia and might not be generalisable to other patient populations. Their analysis included both children and adults, but paediatric patients require a relatively lower dose, which incurs lower
drug costs, which would improve the cost-utility ratio. There was uncertainty concerning the proportionate use of the battery pump versus the balloon infuser for the administration of DFO. Also, equal compliance between the two treatments was assumed, but it is likely that higher compliance would be associated with the oral administration of deferasirox.

Concluding remarks:
On the whole, the study appears to have been based on valid methodology, which enhances the validity of the authors’ conclusions.

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