Cost-effectiveness model for neovascular age-related macular degeneration: comparing early and late treatment with pegaptanib sodium based on visual acuity

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 study assessed the cost-effectiveness of early-, moderate- and late-disease treatment with pegaptanib sodium in patients with neovascular age-related macular degeneration. The authors concluded that treatment with pegaptanib should commence as early as possible to maximise its clinical and economic benefit. Overall the study was based on valid methodology and the reporting was adequate. The authors’ conclusions appear to be appropriate for the analysis undertaken.

Type of economic evaluation
Cost-effectiveness analysis, cost-utility analysis

Study objective
The aim was to compare two options for the treatment of neovascular age-related macular degeneration (AMD) for three groups of patients; those with early, moderate, and late disease. The analysis involved patients aged 65 years or older.

Interventions
The interventions were treatment with intravitreal pegaptanib sodium (Macugen) at a dose of 0.3mg versus sham injection (usual care) administered every six weeks for a period of 54 weeks. Both treatments could be used alone or in combination with photodynamic therapy using verteporfin.

Location/setting
USA/secondary care.

Methods
Analytical approach:
A Markov model was developed to determine the costs and benefits of the two strategies over the patients’ lifetime. The authors reported that the health service payer perspective (third-party payer) was adopted.

Effectiveness data:
The clinical data were primarily derived from two phase II, multi-centre, randomised double-blind studies, that were part of the Vascular endothelial growth factor Inhibition Study in Ocular Neovascularization (V.I.S.I.O.N.). These were supplemented by evidence from administrative databases and other published studies where necessary. Adverse events were considered and these data were derived from expert opinion. The key clinical outcome was the loss or gain in lines of visual acuity.

Monetary benefit and utility valuations:
The patients’ utility values were derived from a published study that employed both time-trade off and standard gamble techniques.

Measure of benefit:
Quality-adjusted life-years (QALYs) and vision-years gained were the summary measures of benefit. These were discounted at an annual rate of 3%.
Cost data:
The direct costs were those of medications, out-patient visits and procedures, adverse event treatment (i.e. endophthalmitis, traumatic injury to lens, and retinal detachment), treatment of possible AMD co-morbidities, and care due to loss of vision (i.e. depression, bone fracture medical care, and care provided in skilled nursing facilities or nursing homes). Apart from out-patient visits, the costs were presented as macro-categories and were mainly calculated using official national sources. The resource use data for treatment-related adverse events, low-vision rehabilitation, and vision aids was based on expert opinion. All costs were in US dollars ($), for the price year 2006. They were discounted at an annual rate of 3%.

Analysis of uncertainty:
One-way sensitivity analyses investigated the uncertainty surrounding the following model parameters: costs and utility values for each health state, clinical efficacy after one and two years of treatment, adverse events probability, risk of death due to total vision loss, treatment efficacy rate after two years, and the discount rate. A worst-case scenario was investigated for pegaptanib in which its efficacy for the first, second, and third year, and the probability of adverse events were varied.

Results
Compared with usual care, pegaptanib resulted in 0.75 additional vision-years and 0.32 QALYs, for early-disease patients; 0.63 additional vision-years and 0.22 QALYs for moderate-disease patients; and 0.22 additional vision-years and 0.09 QALYs for late-disease patients.

The average total costs per patient when treated with pegaptanib were $66,638 for early-disease, $84,185 for moderate-disease, and $96,771 for late-disease patients. The average total costs for usual care were $55,108 for early-disease, $71,393 for moderate-disease, and $84,400 for late-disease patients.

The incremental cost per vision-year was $15,279 for early-disease, $20,350 for moderate-disease, and $57,230 for late-disease patients. The incremental cost per QALY gained was $36,282 for early-disease, $58,280 for moderate-disease, and $132,381 for late-disease patients.

The various sensitivity analyses demonstrated that the results were robust. The incremental cost per QALY gained for late-disease patients was most sensitive to the clinical efficacy in the first and second year, patients' utility, and the costs.

Authors' conclusions
The authors concluded that treatment with pegaptanib should commence as early as possible to maximise its clinical and economic benefit.

CRD commentary
Interventions:
The interventions were clearly reported. The authors acknowledged the limitation that other treatment interventions were not included.

Effectiveness/benefits:
The use of a randomised controlled trial to derive the clinical data was appropriate given the strengths of its design. However, relevant details such as the inclusion and exclusion criteria, and power calculations were not reported. Details of the other studies used were also not reported. This lack of information makes it difficult to objectively assess the validity of the data. The derivation of the benefit measure was briefly reported and it appears that it was based on a validated instrument. The authors used not only a disease-specific measure of benefit, but also QALYs that combine in a single index the dimensions of quality and quantity of life and allow cross-disease comparisons to be made.

Costs:
The costs appeared to reflect the perspective stated. The costs were only presented as macro-categories, but the sources of all data, the assumptions required, the price year, and discounting were extensively reported, which makes the economic analysis more transparent.
Analysis and results:
The costs and benefits were appropriately synthesised by means of an incremental analysis, and the findings were clearly presented. The issue of uncertainty was investigated using a deterministic approach and the key features and the results were appropriately presented. The authors compared their findings with those from other studies and highlighted the possible differences. The authors noted some potential limitations to their study, such as the quality and availability of utility values and the exclusion of indirect costs.

Concluding remarks:
Overall the study was based on valid methodology and the reporting was adequate. The authors’ conclusions appear to be appropriate for the analysis undertaken.

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