Pegaptanib and ranibizumab for treatment of age-related macular degeneration (AMD) – Assessment report

Patient group response

RNIB and the Macular Disease Society appreciate the opportunity to comment on the Assessment report compiled by the Southampton Health Technology Assessment Centre (SHTAC).

Clinical effectiveness

We welcome the statement that "this report has established that ranibizumab is clinically effective for delaying vision loss and improving vision in AMD and that pegaptanib is clinically effective for delaying vision loss associated with AMD." While this appears to suggest that ranibizumab may be superior to pegabtanib we continue to call for both treatments to be made available on the NHS. This is based on the fact - highlighted in the report - that pegaptanib may have disease modifying characteristics and also the fact that there has not been a head-to-head trial to establish the comparative effectiveness of both treatments in terms of improving vision. The report assumes that the risk of vision loss or gain is independent of baseline visual acuity whereas the available data for pegaptanib appears to suggest that patients treated early have a significantly increased chance of vision gain (20%) compared with patients with a lower visual acuity at baseline. In the absence of head-to-head trial results it should be up to clinicians to recommend the most appropriate treatment options to their patients. For instance, clinicians may prefer to opt for

pegaptanib where they are treating patients with a known history of vascular disease.

Cost effectiveness

Having established the clinical effectiveness of both treatments the key question is cost effectiveness. We feel that the results of the cost effectiveness analysis as presented in the report have been superseded by events. The cost estimates used in the model do not reflect the reduction of the price of ranibizumab and the announcement of a different treatment regime that will reduce the average number of injections given to patients. As a result the models need to be re-run using the correct prices and treatment frequency.

In addition, we feel that the costs of sight loss through wet AMD is likely to have been underestimated, even if we accept that NICE is unable to take account of costs that are outside the health and social care sector. We will not revisit the arguments of why we feel that other costs (such as benefits, loss of productivity, informal care, etc.) should be included. These are clearly laid out in our initial submission. We would, however, like to challenge the assumption that the provision of low vision aids and low vision rehabilitation are one-off costs. The gradual deterioration in sight in people with wet AMD means that a low vision device prescribed at the beginning of the sight loss journey may no longer be adequate six months into the journey. Low vision support needs should be assessed on a regular basis - at least annually - to enable patients to receive the most appropriate devices and any mobility training or home adaptations they may need. Also, when seen over a ten year period it is important to recognise that new devices may have been developed that should be made available to all registered blind and partially sighted people, not only those who are recently registered.

In this context we would also like to question the idea that QALY values will drop over time due to patients' ability to adjust to their disability. Whilst it is true that people learn to live with their sight loss to some extent it is also true that their quality of life is not likely to recover substantially. This is due to the social isolation experienced by most people with sight loss and the continuing impact of sight loss on their ability to carry out daily activities. We do recognise that there is insufficient scientific evidence to support this view but feel that our knowledge of the low vision sector bears out these assertions.

Implications for service provision

We entirely agree with the analysis of the implications for service provision of a NICE decision to approve pegaptanib and ranibizumab for use on the NHS. However, we are not clear why the issue of implementation is raised in the assessment report. Clearly NICE's decision should not depend on whether or not NICE believes that there will be capacity problems with the delivery of the service. This may (but should not) have an impact on the length of the period set for implementation and it is certainly an issue that patient organisations as well as specialist commissioners and the Royal College are looking at. However, it should not be included in the assessment report.

Research priorities

Bevacizumab (Avastin)

We are not clear at all why bevacizumab is mentioned in the context of this assessment. Whether or not bevacizumab is as effective as ranibizumab and/or pegaptanib should have no bearing at all on NICE's decision about the cost-effectiveness of the latter two. RNIB and the Macular Disease Society support trials to prove the safety and effectiveness of bevacizumab as long as participating patients are clear that there are alternatives that have been shown to be clinically safe and effective. However, the earliest any bevacizumab trial results are likely to be reported is towards the end of 2008. The inclusion of suggestions to carry out head-to-head trials between the different anti-VEGF trials in this report is not appropriate.

We agree in principle with the other research priorities highlighted in the report but are again not sure why these are included in the assessment report. Any research that is undertaken on the basis of these suggestions is unlikely to have an impact on NICE's decision regarding the cost-effectiveness of pegaptanib and ranibizumab. We would like to see this section removed from the report.

Conclusions

The assessment report is comprehensive and the methodology used is generally sound. Unfortunately, some of the assumptions made and essential elements of the cost figure used need to be revised. It is therefore difficult to come to a final conclusion on the validity of the cost effectiveness data presented in this report.

We look forward to seeing an amended version to inform forthcoming discussions.

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