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

RNIB and Macular Disease Society

Patient group response to the Appraisal Consultation Document issued on 7 December 2007

January 2008
Introduction

1. In this document RNIB and the Macular Disease Society respond jointly to the Appraisal Consultation Document (ACD) sent out on 7 December 2007 to stakeholders participating in the appraisal of pegaptanib and ranibizumab.

2. We welcome the second ACD issued by NICE on the use of pegaptanib and ranibizumab. We are pleased that the responses received to the first ACD from patients, their families and carers and those from the formal consultees have led the Appraisal Committee to amend the initial recommendations.

3. The recommendations made in the second ACD are good for most patients. However, we believe that a number of changes and additions are required to ensure that the Final Guidance will fully meet the needs of the 26,000 people a year who are newly diagnosed with wet AMD. In our response we are calling for:

   3.1. The approval of pegaptanib as second-line treatment
   3.2. A lower treatment threshold with patients being treated in line with the recommendations of the Royal College of Ophthalmologists
   3.3. Clarification of the dose capping scheme
   3.4. The FAD to be issued quickly
   3.5. The speedy implementation of NICE’s guidance and greater efforts by NICE’s implementation unit to monitor and enforce the implementation deadline.
   3.6. Guidelines regarding fast track referral from optometrists/GP to treatment centre.

The decision not to recommend the approval of pegaptanib

4. We continue to believe that clinicians and their patients should have the option to choose what treatment is in the patient’s best interest. For some patients with wet AMD, a selective VEGF inhibitor may be more appropriate, which would make pegaptanib the preferred treatment option. As we have pointed out previously, in reality most patients will be given ranibizumab. Nonetheless a
decision to give pegaptanib on medical grounds should remain a possibility.

The treatment threshold

5. Throughout the ACD a visual acuity of 6/60 is equated with the threshold for legal blindness in the UK. Most significantly the fact that 6/60 is presumed to be the threshold for legal blindness is used as a justification to set the eligibility threshold for treatment at better than 6/60 (effectively 6/48).

6. 6/60 is in fact the threshold for being registered as partially sighted, not blind. The threshold for being registered as blind is 3/60. We believe that the false assumption that 6/60 is the threshold for legal blindness has confused the committee’s thinking. We would like to remind NICE that the eligibility threshold for PDT is 6/60 or better, that the Scottish Medicines Consortium has set no eligibility threshold for ranibizumab and a threshold of 6/60 or better for pegaptanib. Significantly, the Royal College of Ophthalmologists recommends that treatment should be considered until a patient’s visual acuity falls persistently below 6/96 (or logMar 1.2).

7. We hope that with growing awareness of the availability of treatment for wet AMD, increasing numbers of patients will be diagnosed at a relatively high level of visual acuity. However, at present many patients only present with their second eye once they have significant vision loss. Given the chance of improvement in vision through treatment with ranibizumab, these patients should be allowed to access treatment on the NHS. We support the Royal College position and urge NICE to revise its eligibility criteria accordingly.

Clarification of dose-capping scheme

8. We note that discussions between the distributors of ranibizumab and NICE have led to the proposal of a dose capping scheme which will place the financial burden for treatment on the pharmaceutical company after 14 injections. We would want the terms of this scheme to be clear and to be confident that patients who require continuing treatment will receive it for as long as they are likely to benefit.
9. Similar considerations would need to apply if there was a decision to approve pegaptanib based on cost sharing.

**Speedy adoption of FAD**

10. We urge NICE to issue the FAD for this appraisal as quickly as possible. By the time the Appraisal Committee meets again on 13 February 2008 it will have been two years since the draft scope for the appraisal was issued. Because of the delays that occurred throughout the decision-making process hundreds of people will have lost their sight unnecessarily. By issuing Guidance quickly, NICE can ensure that we do not have to add hundreds more to that list.

**Implementation**

11. We believe strongly that the usual three-month period for the implementation of guidance on pegaptanib and ranibizumab should apply. There is no justification for extending this period. Anti-VEGF treatments are being delivered at a large number of centres across England and Wales and as new patients come forward, capacity can be expanded. Experience from the implementation of final guidance on PDT for wet AMD shows clearly that if PCTs and Local Health Boards are given extra time, many will simply delay doing anything for as long as possible. A longer implementation period removes any sense of urgency from their internal decision-making and will again result in unnecessary sight loss.

12. Finally, we would strongly urge the NICE Implementation Unit to work with PCTs and Local Health Boards to ensure that they meet the three month implementation deadline. Since NICE decisions are mandatory NICE itself should take a more active role to ensure the timely implementation of its guidance. As patient organisations we will continue our advocacy work to help patients access treatment and this will include work with PCTs, Local Health Boards and Hospital Trusts. However, we feel that a clear lead from NICE regarding the implementation of its guidance would increase the likelihood that PCTs, Local Health Boards and Hospital Trusts will work together to increase treatment capacity and meet the implementation deadline.
Guidelines regarding fast track referral from optometrists/GP to treatment centre.

13. In the guidance on photodynamic therapy for wet age-related macular degeneration (TA68 issued on 24 September 2003) NICE included the following paragraph about fast-track referrals:

"Wet ARMD can progress rapidly. For a PDT service to be as effective as possible, individuals with early wet ARMD and without serious loss of vision will need to be fast-tracked through the referral and waiting list processes in order to receive treatment before further loss of vision occurs." (p. 15).

14. The importance of rapid referral applies irrespective of the treatment provided. We would therefore like to see the this reference included in the FAD on ranibizumab and pegaptanib.

Final remarks

15. RNIB and the Macular Disease Society have pooled resources to make joint submissions to the NICE appraisal of pegaptanib and ranibizumab. We have been committed stakeholders promoting the interests of the patients we represent. The process has taken longer than expected but we are pleased that we have come this far and can see a positive outcome for the great majority of patients with wet AMD.

16. We very much hope that the NICE Appraisal Committee will listen again and will make the final changes outlined above to bring this process to a satisfactory conclusion.