Appendix G – patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal (STA)

Ranibizumab for the treatment of choroidal neovascularisation associated with pathological myopia

Thank you for agreeing to give us your views on the technology and the way it should be used in the NHS.

Patients and patient advocates can provide a unique perspective on the technology, which is not typically available from the published literature.

To help you give your views, we have provided a template. The questions are there as prompts to guide you. You do not have to answer every question. Please do not exceed the 8-page limit.

About you

Your name: [redacted]
Name of your organisation: Macular Society
Are you (tick all that apply):

- a patient with the condition for which NICE is considering this technology?
- a carer of a patient with the condition for which NICE is considering this technology?
- **X** an employee of a patient organistion that represents patients with the condition for which NICE is considering the technology? If so, give your position in the organisation where appropriate (e.g. policy officer, trustee, member, etc) [redacted]
- other? (please specify)

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General Comments

The Macular Society is a specialist support charity for people with all forms of macular disease. We believe this therapy to be of great benefit to people with myopic CNV and ask NICE to approve it and make it available at the earliest possible opportunity.

What do patients and/or carers consider to be the advantages and disadvantages of the technology for the condition?

1. Advantages
(a) Please list the specific aspect(s) of the condition that you expect the technology to help with. For each aspect you list please describe, if possible, what difference you expect the technology to make.

Loss of central vision can have a devastating affect on a person's life. The macula is the central and most sensitive part of the retina. It has a high density of photoreceptor cells and is responsible for much of our colour vision and the fine detail of what we see. Damage to the macula causes loss of colour perception, blurring or distortion of vision and finally frank gaps in the central vision where scotomas have formed. As a result, a person with a damaged macula cannot drive, read, see what food is on their plate or recognise the faces of their family and friends. Many will qualify to be registered as severely sight impaired (blind).

For people of any age the condition can lead to isolation, loss of independence, increased level of falls and other accidents as well as depression, often severe. For many people with myopic maculopathy, who are usually younger than the age-related macular disease population, there are the additional aspects of loss of employment and the prospect of an impoverished future as well as the inability to attend to family responsibilities such as caring for children.

There is currently no other effective, licensed treatment for the overwhelming majority of these patients. Unlicensed anti-VEGF drugs, both ranibizumab and bevacizumab have shown considerable benefits for those able to access them.

These are reported in the manufacturers' REPAIR trial and other trials.

Intravitreal ranibizumab for the primary treatment of choroidal neovascularization secondary to pathologic myopia.
Lai TY, Chan WM, Liu DT, Lam DS**.**
Retina. 2009 Jun;29(6):750-6.

Intravitreal ranibizumab (Lucentis) for the treatment of myopic choroidal neovascularization.
Konstantinidis L, Mantel I, Pournaras JA, Zografos L, Ambresin A.

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Ranibizumab for the treatment of choroidal neovascularisation associated with pathological myopia

Graefes Arch Clin Exp Ophthalmol. 2009 Mar;247(3):311-8. Epub 2008 Nov 29.

Short-term efficacy and safety of intravitreal ranibizumab for myopic choroidal neovascularization.
Silva RM, Ruiz-Moreno JM, Nascimento J, Carneiro A, Rosa P, Barbosaa A, Carvalheira F, Abreu JR, Cunha-Vaz JG.
Retina. 2008 Oct;28(8):1117-23.

Retina. 2009 Jun;29(6):750-6.

(b) Please list any short-term and/or long-term benefits that patients expect to gain from using the technology. These might include the effect of the technology on:

- the course and/or outcome of the condition
- physical symptoms
- pain
- level of disability
- mental health
- quality of life (lifestyle, work, social functioning etc.)
- other quality of life issues not listed above
- other people (for example family, friends, employers)
- other issues not listed above

Anti-VEGF therapy for patients with CNV as a result of high myopia can be expected to:

- improve or stabilise vision and prevent loss of vision (which without treatment is inevitable) thus very significantly reducing the level of disability
- reduce the incidence of depression in people who would not now lose their sight
- reduce the incidence of falls and other accidents which occur in visually impaired people
- maintain an individual's employability and their and their family's financial wellbeing so reducing poverty and the need for state benefits
- maintain and individual's quality of life
- enable an individual to take a full part in family, social and cultural life
- enable an individual to maintain family and caring responsibilities for example for children
- reduce the burden of caring and anxiety on family and friends

NHS Cambridge and Peterborough published a Position Statement on this subject in May 2012 and explained the advantages of the treatment thus: "There is no treatment currently available for the vast majority of these patients. An occasional patient might be amenable to treatment by laser photocoagulation or verteporfin photodynamic therapy; though both of these treatments have significant adverse effects due to their destructive mechanism of action and the outcome is usually not very good. In short, most of these patients become blind under current management while most will maintain or improve vision with anti-VEGF therapy." In spite of this bluntly expressed judgement of the value of anti-VEGF therapy it is withheld from patients in many part of the country; many but not all. Availability of this treatment is currently a 'postcode lottery'. Patients

find this very cruel and it adds to their psychological distress. Making this therapy available across the NHS would greatly benefit patients who are currently refused it.

2. Disadvantages
Please list any problems with or concerns you have about the technology. Disadvantages might include:
- aspects of the condition that the technology cannot help with or might make worse
- difficulties in taking or using the technology
- side effects (please describe which side effects patients might be willing to accept or tolerate and which would be difficult to accept or tolerate)
- impact on others (for example family, friends, employers)
- financial impact on the patient and/or their family (for example cost of travel needed to access the technology, or the cost of paying a carer)

Anti-VEGF injections are considered safe and give rise to relatively few side-effects (compared with laser or PDT for example). The most common disadvantages of anti-VEGF injections are inconvenience at having to have repeated injections, pain or discomfort in the eye, inflammation and occasional infection.
Myopic CNV appears to require fewer injections than needed in patients with wet AMD so all side effects are reduced compared with wet AMD. In our experience none of the side effects is likely to dissuade patients from having the treatment.

3. Are there differences in opinion between patients about the usefulness or otherwise of this technology? If so, please describe them.
None of which we are aware.

4. Are there any groups of patients who might benefit **more** from the technology than others? Are there any groups of patients who might benefit **less** from the technology than others?
Patients with less severe disease or those more recently diagnosed may benefit more from the technology.

Comparing the technology with alternative available treatments or technologies
NICE is interested in your views on how the technology compares with existing treatments for this condition in the UK.
(i) Please list any current standard practice (alternatives if any) used in the UK.

There is no licensed alternative to this technology. Bevacizumab has been used in many places where commissioners have refused individual funding requests for ranibizumab or where the patient is paying for private treatment.

(ii) If you think that the new technology has any **advantages** for patients over other current standard practice, please describe them. Advantages might include:
- improvement of the condition overall
- improvement in certain aspects of the condition
- ease of use (for example tablets rather than injection)
- where the technology has to be used (for example at home rather than in hospital)

- side effects (please describe nature and number of problems, frequency, duration, severity etc)

There is no other effective licensed treatment for this condition currently available.

(iii) If you think that the new technology has any **disadvantages** for patients compared with current standard practice, please describe them. Disadvantages might include:
- worsening of the condition overall
- worsening of specific aspects of the condition
- difficulty in use (for example injection rather than tablets)
- where the technology has to be used (for example in hospital rather than at home)
- side effects (for example nature or number of problems, how often, for how long, how severe).

None.

Research evidence on patient or carer views of the technology
If you are familiar with the evidence base for the technology, please comment on whether patients' experience of using the technology as part of their routine NHS care reflects that observed under clinical trial conditions.

Patients to whom we have spoken appear to benefit from the treatment in line with the findings of the REPAIR study.

Are there any adverse effects that were not apparent in the clinical trials but have come to light since, during routine NHS care?

None of which we are aware.

Are you aware of any research carried out on patient or carer views of the condition or existing treatments that is relevant to an appraisal of this technology? If yes, please provide references to the relevant studies.

No formal research of which we are aware. Anecdotal evidence has come to us from our helpline and advocacy services which have received many calls from patients who are desperate to receive this treatment.

While some patients have been recruited into trials in recent months, over the last five years our advocacy service has provided general information and advice to many patients with myopic CNV and has directly supported the individual funding applications of 32 patients. Some applications have succeeded and others have failed. There is no obvious reason for the difference in the decisions. The application process is very stressful for patients. Patients' main concerns as expressed to the advocacy officer are:
- having to fight for treatment. Without exception callers are upset that, at a relatively early age, they are being refused treatment and the chance to keep their sight when other people in their 80s or 90s (with wet AMD) are treated automatically. This is

especially upsetting as the patient is likely to have been told by their ophthalmologist that there is a treatment available which the consultant believes would be of benefit.
- fear that they will lose their sight before they are allowed treatment. Patients know that treatment has to be given quickly to be effective. They are very anxious that precious time is being lost while the individual funding application is made and perhaps refused and appealed.
- not being able to work and so being unable to pay the mortgage and/or work towards a pension. Even if it is one eye which is affected, patients are in great fear that the second eye will develop the condition.
- not being able to help their children in ways which will disadvantage their children in later life for example not being able to help with homework.
- fear that their children will also get the condition.
- anger that they are being 'let down' by the 'system'.

Callers often describe experiencing great fear, of having lost their whole way of life and of having no idea how to cope. In our experience patients are extremely distressed by this condition and are often desperate to have access to this therapy to retain their sight.

Availability of this technology to patients in the NHS
What key differences, if any, would it make to patients and/or carers if this technology was made available on the NHS?

The technology would significantly reduce disability in this group of patients benefiting the patients, their families and, often, their employer.
It will help meet the Government's stated aim of reducing avoidable sight loss as set out in the public health indicators.

What implications would it have for patients and/or carers if the technology was **not** made available to patients on the NHS?

Patients will lose their vision unnecessarily. With this comes a long list of economic, social, psychological and physical problems including:
- unemployment
- loss of independence
- reduced quality of life
- inability to perform family/caring responsibilities
- inability to participate fully in social, cultural or recreational pleasures
- poorer mental health including anxiety, depression and severe depression
- poorer physical health including the higher risk of injury and accident and the poorer health associated with poverty.

Sight loss is a major cause of depression when it cannot be treated. We believe patients suffer psychologically even more when a good treatment exists but they are denied access to it.
In the absence of NICE guidance on this condition, patients are subject to a postcode lottery in which some will get treatment and others won't, depending on the views of their local commissioning body. Commissioners often claim that they are justified in refusing treatment for Myopic CNV as it is not covered by NICE guidance TA155

(Lucentis for wet AMD). This use of the absence of NICE guidance as a reason for refusing funding off-label treatments has been common even when treatment has been recommended by clinicians.

Some patients will make significant financial sacrifices to pay for their own treatment when it is refused. Others do not have the resources to pay for private care. A lack of NHS approved treatment thus contributes to the health inequalities in the UK.

Are there groups of patients that have difficulties using the technology?

It is possible that people who have a phobia of needles or severe physical or mental disability might find it difficult to use this technology.

Equality
NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that this appraisal:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which [the treatment(s)] is/are/will be licensed;
- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;
- could lead to recommendations that have any adverse impact on people with a particular disability or disabilities.

None

Please tell us what evidence should be obtained to enable the Committee to identify and consider such impacts.

Other Issues
Please consider here any other issues you would like the Appraisal Committee to consider when appraising this technology.

The views of 2 patients who contacted our advocacy service.

Patient 1.

This patient is 58 years old. She is a self-employed translator and proof reader for which her sight is essential. Five years ago she experienced 6 months of distorted vision and then significant loss of vision in her right eye before being diagnosed with myopic CNV. Her consultant ophthalmologist applied to the local primary care trust for funding for anti-VEGF therapy but was refused and an appeal was also rejected. Subsequently, the patient paid privately for 9 Avastin injections to preserve the vision in her right eye. The first two injections resulted in much improved vision and the other injections have enabled her to retain the sight in her right eye. She was later

enrolled in a trial and has received a further 5 injections as part of the trial. Since the end of the trial she has had no further treatment.

The patient describes her experience of myopic CNV and her attempts to get treatment as “extremely stressful”. She has been very worried that her left eye might develop CNV as well (she has high myopia in both eyes), putting her livelihood at risk. She has spent many thousands of pounds on private treatment and says she worried constantly about where the money would come from to pay for the next injection. She found it “extremely upsetting” that she was refused NHS care. She says she has paid into the NHS all her life and the first time she really needed NHS care she was refused. The distress of this was made worse by the knowledge that other people with the exactly same condition were getting NHS care. The patient had personal knowledge of a friend in another part of the country with the same condition whose PCT approved NHS treatment. This unfairness caused the patient considerable distress.

The patient lives with the fear that the CNV will reactivate and that she will have to find yet more money to retain her sight.

Patient 2.
This patient is 45 years old and a building control surveyor for a local authority. He has four children. He lost the sight in his left eye at the age of 14 when he suffered a severe haemorrhage and has only light perception in that eye. He developed myopic CNV in his right eye 7 years ago. He underwent laser treatment and photodynamic therapy but these did not resolve the CNV. His consultant applied to the local PCT for funding for anti-VEGF therapy but this was refused. He was treated with Avastin some three months later but is not sure how that treatment came to be approved.

Since then he has had 2 further injections and his vision has been stable for 2 years.

The patient found the experience of being refused treatment and the prospect of blindness terrifying. He feared he would lose his job and be reliant on state benefits for the rest of his life. He felt extreme anger at the PCT officials who refused him treatment and questions whether they would have done the same if it was their own child who needed the treatment.

He described his feelings: “This was the worst point of my life, ever. I only had one eye left and I thought; is this it? Am I going blind? I felt frustration, anger, turmoil and total desperation. I couldn’t understand how they could do this to someone for the sake of a £100 injection [the cost of a vial of Avastin although the patient understands there are other costs involved as well]”.

He has had a cataract replacement in his right eye more recently and now can drive again. He says that his mental wellbeing is the best it has been for 7 years.