Appendix G – Patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Lenalidomide for the treatment of myelodysplastic syndromes associated with a deletion 5q cytogenic abnormality in people with red blood cell transfusion dependence [ID480]

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: Prof Rodney Taylor; [redacted]

Name of your organisation: MDS UK Patient Support Group & Leukaemia CARE (joint submission)

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?
- an employee of a patient organisation 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)

x other? (please specify) MDS patient and chairman (volunteer) of a patient organisation; Chief Executive (volunteer) of the patient organisation

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Lenalidomide for the treatment of myelodysplastic syndromes associated with a deletion 5q cytogenic abnormality in people with red blood cell transfusion dependence [ID480]

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.

Advantages for patients and family members:
Generally, vastly improved Quality of Life (QOL) – by providing transfusion independence (TI)

Slowing down of disease progression

Stabilisation of blood counts – especially haemoglobin levels – meaning freedom from regular ups and downs of severe fatigue levels between transfusion intervals, as well as feelings of depression and anxiety which often occur when patients are not able to contribute to normal household tasks.

Lenalidomide is a treatment for MDS – which changes the nature of the disease as opposed to mere supportive care.
It therefore cannot and must not be compared to transfusions which merely assist patients to supplement their low haemoglobin levels – and nothing else.

No danger of iron overload due to frequent transfusions – and none of the health issues associated with iron overload.

No need for additional treatment of iron chelation – as no risk of iron overload associated with regular transfusions in low-risk MDS. Additional cost saving for NHS.

None of the risks and complications associated with frequent transfusions.

Oral treatment as opposed to invasive and sometimes risky procedure of transfusions.

No need for frequent trips to hospitals – with associated time constraints, costs of travel, often also need to be accompanied by family member

If patient still in work – no need for frequent time off work for blood transfusions and at hospital.

Ability to return to work, to travel, to contribute to household work – basically enjoy an almost normal way of life.

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Lenalidomide for the treatment of myelodysplastic syndromes associated with a deletion 5q cytogenic abnormality in people with red blood cell transfusion dependence [ID480]

Should patients consider a bone marrow transplant in the future, a low ferritin level is advisable for a successful outcome – therefore best to avoid repeated transfusions.

(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

Lenalidomide is potentially slowing down the progression of MDS.
The main advantage is the blood transfusion independence it offers the patients – therefore vastly increasing their quality of life.

Blood transfusions are extremely disabling for the following reasons:
- requiring frequent and tiring trips to hospital – often accompanied by family member
- extreme fatigue and short bursts of energy – between transfusion intervals
- feelings of depression, anxiety due to inability to perform normal activities or participate in daily household tasks
- inability to work – as fatigue usually too severe
- previously independent elderly patients gradually necessitating carers as unable to safely look after themselves
- reliance on family members for help – who often have to take time off work to accompany patient to hospital appointments.

This is an oral drug meaning fewer trips to hospitals – presenting a substantial saving of time, costs of trips and cost of parking at hospitals – cost savings for the NHS, the patients themselves and therefore also social services.
Transfusions can take up to 8 hours in hospital – as frequently as every 2 weeks.
An oral drug avoids the issues sometimes associated with regular transfusions and low haemoglobin – such as trouble finding a vein, painful transfusions, repeated invasive needle pricks.

Being transfusion free is now known to improve survival time.
This in turns helps with feelings of severe anxiety associated with the diagnosis of MDS.

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Lenalidomide for the treatment of myelodysplastic syndromes associated with a deletion 5q cytogenic abnormality in people with red blood cell transfusion dependence [ID480]

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 thier family (for example cost of travel needed to access the technology, or the cost of paying a carer)

Very few and minor disadvantages – compared with the enormous advantages this technology offers.
Need to take an oral every day.
None of the patients we know have reported specific disadvantages.

3. Are there differences in opinion between patients about the usefulness or otherwise of this technology? If so, please describe them.

Sometimes patients hesitate to start treatment with Lenalidomide – as with every other regular treatment – out of habit of having transfusions and fear of the unknown. This is normal and often the case with new treatments.
However – all patients on Lenalidomide we are aware of are extremely happy with the improvements this drug has had on their quality of life.

Also the knowledge that this drug is treating their MDS is vastly improving their outlook on the future regarding their survival chances and hope for an extended survival time.

See also Appendix 1 – additional Lenalidomide patient statement.

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?

All transfusion dependent del 5q patients would benefit from this drug.
No exceptions.

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Lenalidomide for the treatment of myelodysplastic syndromes associated with a deletion 5q cytogenic abnormality in people with red blood cell transfusion dependence [ID480]

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 other comparable treatment for del 5q MDS.

Supportive care is currently offered to those patients – which is not an accurate comparison for Lenalidomide.
As with the NICE appraisal of azacitidine (2009-2011) – it is extremely important to clearly understand MDS and not rely on supportive care as the only comparator.
(MDS UK 2.2 Misunderstanding of myelodysplastic syndromes led to a perverse reliance on best supportive care as the only comparator.
http://www.nice.org.uk/nicemedia/live/12036/53140/53140.pdf)

Lenalidomide treats the del 5q and positively influences the nature and progression of the disease – whereas transfusions/supportive care merely offer a temporary top-up of haemoglobin or assistance with infections.

Repeat platelet transfusions can lead to growing intolerances and rejections, and the further complication of having to find new matches.

Costs of transfusions are vastly underestimated – as they do not take into account hospital staff time, hospitalisations due to infections or reactions to transfusions and associated drug treatment.
Cost effectiveness, as always, is related, not only to the costs of providing the drug, but also the costs of transfusions and the substantial amount of non productive, patient time time associated with the transfusions, typically 2.5 hours per unit of blood transfused.

The option of a bone marrow transplant is not a suitable comparator either – as too few del 5q patients are able to sustain such a transplant – due to their age and concomitant conditions.

Lenalidomide is truly a unique treatment option and absolutely needs to be offered to del 5q patients.

(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)

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Lenalidomide for the treatment of myelodysplastic syndromes associated with a deletion 5q cytogenic abnormality in people with red blood cell transfusion dependence [ID480]

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

Lenalidomide presents an enormous advantage over the current standard practice of supportive care.
Lenalidomide potentially slows down the progression of the condition, offers transfusion independence, and potentially extends survival time.

Lenalidomide is taken orally as a tablet – which is a most convenient mode of administration – in comparison to invasive transfusions. It is taken at home.

The patients we are aware of do not report any significant side-effects.

Lenalidomide is a disease modifying drug – as opposed to the temporary measure of blood transfusions which lead to a definite and slow deterioration of health.

(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).

Some research results are still debated on whether Lenalidomide speeds up the progression to AML. At time of this submission, the opinions are still divided but the opinion is that the patients who did progress to AML in trials were more severe in the first place.

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.

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

None.
Lenalidomide has not been offered as routine NHS care so far.

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Lenalidomide for the treatment of myelodysplastic syndromes associated with a deletion 5q cytogenic abnormality in people with red blood cell transfusion dependence [ID480]

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.

Yes – we have attached 3 studies about quality of life in MDS patients – as well as the importance of transfusion independence in MDS patients.
These 3 papers have been included and accepted as evidence in the previous NICE appraisal of azacitidine in 2010.

Also attached – the testimony of a del 5q patient who has benefitted from lenalidomide for several years.

We are aware of numerous other patients with equally successful outcomes.

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?

Lenalidomide would represent an enormous advantage to patients, families and carers.
Differences regarding:
- Effect on fatigue due to a steady haemoglobin level
- Effect on survival time
- Patient's mental health
- Family's mental health
- Patient's contribution to family life, society and often work life

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

Higher need of NHS blood products
Higher need of hospital visits, staff time
Higher number of transfusion reactions and complications – potentially in-patient stay
Higher incidence of iron overload problems
Higher need for iron chelation drugs
Higher number of deaths of del 5q patients
Higher dependence on state help, social costs

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

None

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Lenalidomide for the treatment of myelodysplastic syndromes associated with a deletion 5q cytogenic abnormality in people with red blood cell transfusion dependence [ID480]

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.

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.

Patient population affected is extremely small.
We estimate that 500 del 5q patients per year would need Lenalidomide – therefore the impact on the overall health budget would be fairly minimal.

Blood transfusion costs are vastly underestimated – not only the administration of transfusions, but also the cost of treating the complications attached to those transfusions. These include:
- the cost of in-patient treatment for repeat infections
- cost of additional blood matching when patients can no longer tolerate further platelet transfusions of the same type
- cost of iron-chelation therapy

See documents in Appendix:

Appendix 1 – Lenalidomide patient experience

Appendix 2, 3, 4 – Papers pertaining to the importance of transfusion independence.
All 3 documents were accepted as evidence in the NICE appeal meeting of azacitidine in July 2010. The appeal panel agreed these documents should have been consulted from the start of the appraisal.