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

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: **Fiona Pirilla**

Name of your organisation: **MDS Patient**

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)
- other? (please specify)

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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.

- **Tranfusion independence** – if I was not taking Lenalidomide I would undoubtedly be having regular blood transfusions.

- General health improvement – my haemoglobin counts are vastly improved which has totally relieved my previous symptoms of anaemia. This has allowed me to return to participating in exercise and generally improved my overall health. I now feel I lead a normal life, instead of feeling ill most of the time.

- **Ability to work** – when my haemoglobin counts were starting to get very low I found it increasingly difficult at work. As a Health and Safety trainer my job generally involves standing up all day long running training courses, which also relies on a level of personal energy to deliver the courses and interact with the delegates. My job also requires a lot of driving around the whole UK and occasional travel abroad. I had to discuss my increasing fatigue levels with my employer as my job was becoming increasingly harder due to this. They were able to work with me to allow me to cut down to working only 4 days per week. The advantage of now being on this treatment is that my condition doesn't affect my work at all. I could return to working 5 days a week again. I haven't done this, but this is now due to my personal choice rather than my health. I believe that if I had not been on this treatment I would possibly have had to cut down my working week even more, or even leave that job altogether and try and find something else less demanding. When a prospective employer asks you why you want to leave your current job, I'm sure they would undoubtedly have been put off employing me once I told them about my health issues. Now I feel totally able to cope with my workload, the long working days, the long driving hours, and my ability to cope with stress is greatly improved because I don't feel the emotional strain of being constantly fatigued all of the time.

- **Less time spent in hospital –** both due to having less hospital visits generally (at one point I was having to have blood tests every week, whereas now I only need to go every 2 months, with pregnancy tests in-between).This is a massive advantage for me personally and also my employer as I need less time off work to do this. Having blood transfusions takes all day and I wasn't always able to schedule this for my day off. Even my normal visit to a consultant at my local hospital takes typically 2-3 hours once I've had my blood test, seen the consultant and gone to the pharmacy, but now the frequency of these visits is much less. My visits to Kings College Hospital in London, where I originally participated in the drug trial were every month, so as you can imagine this again would require a whole day off work and also the cost of travelling down to London. Now my local hospital is able to prescribe the drug which

means it is much more convenient and cost effective for me personally. I now only need to visit Kings College hospital once every 6 months.

- **Slows down the progression of the disease** – Although my disease could still progress I am currently fairly stable and have lived with this disease since being diagnosed in 1999. Without the drug, my disease would surely have progressed at a faster rate.

(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
Currently my treatment using this drug helps to maintain the stability of my condition and takes away the need for other time consuming treatments such as blood transfusions. I know that it is not a cure so to speak but it definitely seems to have slowed down the progression of my illness.

- physical symptoms
As a result of this treatment I no longer suffer with the symptoms of anaemia, as my haemoglobin results are within the "normal" range. I don't in any way suffer the same level of fatigue, breathlessness, and feeling cold all the time as I have done in the past and am less susceptible to infections. When I was initially diagnosed with MDS I had a really bad chest infection which wouldn't go away and required several GP visits and eventually visits to 2 different consultants at hospital. This eventually led to my diagnosis of MDS. During this time I had required several doses of anti-biotics and was put on three different types of asthma inhaler and was also given steroids to try and calm down my coughing. During one particularly violent coughing fit I actually cracked a rib!. Prior to treatment with Lenalidomide I was often susceptible to colds and infections, particularly chest and water infections, and these would often require antibiotic treatments. Thinking back now it has been well over 15 months since I have had to go to a doctor and have antibiotics, and the last time it was for Campylobacter food poisoning, but I've not required anything since then.

- pain
I have no real experience of this as I've never really experienced pain with this condition, other than when I was still playing basketball (for England) where the anaemia had a massive effect and made it very difficult to breathe during exercise and recover afterwards.

- level of disability
Prior to taking this drug, I was forced to stop playing basketball, at first for England, and then for my club teams too because it was physically too demanding. This initially made me feel much better, but eventually through progression of the disease, the symptoms then started to affect my daily life in a derogatory way. Now however the drug means I can return to "normal" levels of exercise – too late for me

to start playing basketball again, but ok for "normal" exercise such as going to the gym and long walks etc.

- mental health
I no longer feel the emotional effect of being exhausted all the time and the toll it takes on my work and home life. I feel less stressed out because of this and more optimistic about my future because I feel so much better. I don't think I originally realised the affect on my home life because you are so consumed with how unwell you feel you don't necessarily think of the affect on others. I think that I was often irritable and snappy due to constant fatigue and my husband in particular would have borne the brunt of this! I am not by nature a negative person but I have definitely experienced moments of uncertainty and times of feeling down about what the future holds. This treatment at least gives me the hope of slowing down the progression of my disease long enough to hopefully avoid other riskier treatments such as bone marrow transplants and hope for the ongoing development of other drug treatments.

- quality of life (lifestyle, work, social functioning etc.)
I feel totally back to normal in my life generally. I know that I have a serious illness, but it's not at the forefront of my thinking anymore because I live a full and normal life again. I don't feel restricted at all any more from a quality of life viewpoint. I can participate in all aspects of my life again normally both at work and outside of work with my friends and family. Most people looking at me now would have no idea from looking at me that I have a serious illness. The only reason for that is the treatment that I now have. Without it, the symptoms of anaemia would definitely show much more physically and giving me the appearance of a person who is ill. Thankfully I don't have that appearance now of looking very pale and washed out, I look healthy and normal.

- other quality of life issues not listed above
It is very difficult to plan your life ahead with a disease such as MDS because the future is always uncertain. My husband and I got married three years ago when I was not receiving treatment. This was during a period of time where I had participated in the initial drug trial (from September 2006 – December 2007) but then had not continued to receive the drug after the trial had finished. As my condition again worsened, I was finally given access to the drug the second time from January 2010 onwards. My disease was not one of the reasons why we got married, but I now have comfort from the fact that I am happy all my financial and legal aspects are in order due to this should anything ever happen to me.
With MDS I found that my condition deteriorated slowly to begin with and initially you just try to adapt and don't always realise how ill you really are until you feel better. When I was originally on the drug trial my health improved dramatically and when I finished the drug trial and then had that long period of time without the drug it was very emotionally and physically hard for me. I knew how ill I'd felt before the drug and I knew how much better I had felt with it. I then had to cope with feeling myself slipping back again because I wasn't taking it. This was a very emotional time for me because I knew exactly where my health was headed again and it wasn't a happy time for me because I felt I had no options.

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- other people (for example family, friends, employers)
I feel that the quality of relationship I have with everyone now, is much improved due to the improvement in my health. I initially dropped down to working 4 days a week because of my health because I was so exhausted. Even though my health has improved I have continued to only work 4 days a week but the difference is that this is now due to personal choice and not necessity. I would certainly be fit enough to work 5 days again, and do from time to time when my company requires it. I have now been able to take on more aspects of my job and work for longer both in terms of potential number of days that I can work and also the hours. My career progression would undoubtedly have been affected a few years back, but now is back within my control. When I felt exhausted all the time it would make me very emotional, and often snappy and irritable with people, and many of my friends, family and work colleagues have commented on this improvement.
- other issues not listed above.

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

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.
Essentially I understand the fact that this drug is not a cure and that we don't necessarily fully understand the long term aspects of treatment with it in this condition. I fully accept and am prepared to acknowledge that risk in order for the health benefits I receive now from the treatment.

- difficulties in taking or using the technology
I can honestly say that there have been no difficulties at all for me. It is simply a case of taking an oral tablet every morning.

- side effects (please describe which side effects patients might be willing to accept or tolerate and which would be difficult to accept or tolerate)
The only side effect I experience is that the drug treatment reduces my white cell and neutrophil count. I take regular GCSF injections to counteract this but this can cause some difficulties when travelling as these need to be refrigerated at all times. I have always managed to overcome these problems when working in the UK, but it can be more problematic when travelling abroad. I experience no difficulty in administering the injections myself, but appreciate that other patients may.

- impact on others (for example family, friends, employers)
I've experienced no disadvantages at all other than the fact that MDS sometimes restricts me on countries that I might need or like to visit (because of a lowered

immune system) but this is a side effect of the condition generally and not due to the treatment.

- 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).
Again no disadvantages at all as this treatment means I spend less time being treated and more time getting on with my life relatively normally. Fewer hospital visits means less cost and inconvenience for me. Because the treatment has now cut down on my time spent at hospital there is a cost saving for me with regards to hospital car parking, travelling to hospitals especially when I was having to travel to London. My visits to London were often on the train, which can be expensive and even when I drive fuel costs today are very high, so less visits is a big saving.

3). Are there differences in opinion between patients about the usefulness or otherwise of this technology? If so, please describe them.
Personally as a patient, I have only ever met one other person that took this drug, and he also had a very positive response.

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?
I am not a medical person, but I would imagine that most people with MDS 5q- would benefit from this treatment. As I was only in my thirties when I was originally diagnosed, it has made a massive difference to me. I think there are benefits for patients of any age, but particularly if you are young, like I am.

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.
-" Watch and wait" – which is what I was doing initially
- Blood Transfusions or other supportive drugs to raise haemoglobin levels. Blood transfusions are only really a temporary measure to top up haemoglobin levels and only have a very short term effect. This in no way compares to the benefit I now receive from taking the drug because the beneficial effect with transfusions was only ever short lived, and in no way altered the progression of my condition in the way that this drug treatment does.

(ii) If you think that the new technology has any **advantages** for patients over other current standard practice, please describe them. Advantages might include:

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- improvement in the condition overall
From personal experience when I was on the original drug trial my health dramatically improved and without this drug I would definitely be transfusion dependant and my disease would have progressed further.

- improvement in certain aspects of the condition
I would say ALL aspects of my condition have now improved because of this treatment because of the increase in my haemoglobin count. . My anaemia improved greatly because my haemoglobin levels shot back up. This greatly improved my fatigue levels and made me feel less washed out, cold and pale. My frequency of infections has now also greatly reduced.

- ease of use (for example tablets rather than injection)
The drug is very easy to take because it is in tablet format. I do have to use GCSF injections also, but find these easy to manage.

- where the technology has to be used (for example at home rather than in hospital)
This has certainly resulted in less general consultant visits, and has also meant I don't have to spend all day in hospital having a transfusion. Although I had only needed a few before I actually went on the trial, they were very time consuming and inconvenient both for myself and my employer too. When travelling to Kings College in London for initial consultation visits my husband would often try to come with me, but this wasn't always possible if he couldn't get time off work. Less visits means not only I benefit, but so does he in terms of the amount of time and cost for him.

- side effects (please describe nature and number of problems, frequency, duration, severity etc.)
I've never really suffered with any major side effects from the drug treatment. I have mentioned the side effect that the drug treatment has of lowering white cell and neutrophil counts but that I take the GCSF injections to counteract this. I currently need to administer these every other day but these are really quick and easy for me to do and don't create any issues other than when travelling.
In 2011 I did have phlebitis in my left arm which caused it to swell slightly. I believe this is probably due to having so many blood tests in the past and deterioration of the vein in that arm. Scans showed no signs of any clots, and I continue to take an aspirin every day and have not had any further problems related to this. I now generally have blood taken from my right arm instead.

I believe that I have always been kept abreast of any potential side effects of the drug through information I was always given during the drug trial and since. These potential risks for me far out way the benefit I receive from taking the drug.

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(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).

Again just speaking personally, I can't think of any disadvantages at all compared with the standard supportive care.

Equality and Diversity

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
I'm not entirely sure how to answer this question therefore have left it blank.

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.
There is no current routine use of this drug for MDS as yet therefore I felt unable to answer this question.

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

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Yes – when my initial drug trial ended in 2007 I was then unable to gain access to the drug until 2010. During this three year period I had to undergo the process of trying to get funding from my local PCT who turned me down because the drug wasn't licenced. During the drug trial my response was excellent both with the blood counts and bone marrow and cytogenetics. The adverse affect I had once this trial stopped was that my health started to deteriorate again, and it took me nearly three years to be able to obtain this treatment again. The stress of this as you can imagine was great, to say nothing of my deteriorating health again. After starting the drug again in January 2010 and now remaining on it ever since, my blood counts have improved again, but my bone marrow has not necessarily reacted in the same way and my disease still shows progression. I will never know how much effect my three year absence of the drug has had on my condition, and feel very strongly that there should be a clear pathway for patients that have responded positively during drug trials to enable them to continue successful treatments when the trials finish and not be left in limbo, as I was.

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.

I believe that the UK MDS Patient Support Group has already supplied 3 papers in reference to this.

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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?
I think this drug has the potential to make a massive difference to people with MDS 5Q- if people respond in the way that I did. An overall improvement of health and symptoms, enabling a much fuller and more normal life to be lived without feeling unwell all of the time and being prone to infection.

Not only does it affect the physical aspects of a patient, but also the emotional side. This treatment gives hope to people and almost makes you forget you have an illness and allows you to live and work completely normally again.

The whole aspect of my health returning to "normal" means that I can also work normally too without having the worry of if I will ever have to cut down or stop work altogether and the whole financial implication that would have for me and my family.

There would also presumably be a big difference to the NHS resources also in terms of less time spent treating patients both in appointments and clinics.

What implications would it have for patients and/or carers if the technology was **not** made available to patients on the NHS?
Potentially a shorter lifespan for some people and a continued drain on resources both for patients, families and the NHS.

Are there groups of patients that have difficulties using the technology?
I don't believe so.

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Other Issues

Please include here any other issues you would like the Appraisal Committee to consider when appraising this technology.
I think that in the grand scheme of things the small number of MDS transfusion dependant patients means that the overall impact on the NHS health budget of providing this drug would also be small.

It would also have the benefit of saving our national blood resources for other patients that need them, whether that be for an existing condition or emergency situations. This drug can potentially totally negate the need for blood transfusions in MDS 5q- patients and therefore save that precious resource.

Long term transfusion dependant patients will also be saved from the associated health issues of regular transfusions, such as iron overload for example. Reducing these issues will reduce the burden on the patient but also the burden on the NHS to treat this too.

My final point would also be that as a patient in the UK it is very difficult to accept that my fellow patients across the Atlantic in the USA have had access to this drug for a while now and yet it is not accessible here? Surely we should be able to share the benefits of experience and information in other countries and work together with the drug companies as much as possible so that a practical and cost effective solution can be found to benefit patients in the UK too.