Patient/carer organisation statement template

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] **-** [redacted]

Name of your organisation: MDS UK Patient Support Group
Joint statement with The Leukaemia Society (UK).

Are you (tick all that apply):

- **X a patient with the condition for which NICE is considering this technology?**\
[redacted] **(**[redacted]**)**

- a carer of a patient with the condition for which NICE is considering this technology?

- **X 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)**

[redacted] (Leukaemia Society)

- other? (please specify)

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.

1. Patient quality of life: MDS patients live with cytopenias and a continuous need for blood transfusions due to anaemia. This need for transfusions causes patients to suffer a severe decrease in their quality of life since they must be able to obtain a transfusion when needed, they are required to visit their physician or the hospital for blood tests, and to spend a significant amount of time actually receiving the transfusion.

VIDAZA (azacitidine) eliminates the need for transfusions in a majority of patients with MDS. Fatigue is lessened with the increase in red blood cells produced in the bone marrow. These two things increase patients quality of life.

2. Increased survival. MDS patients were shown, in the pivotal trial upon which approval was based in Europe, to have an increased median survival of more than 9 months. There is no cure for MDS – except BMT – and this drug provides patients with their best hope for a longer life.

(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.

1. Improved survival – increase in median survival of >9 months
2. Relief of debilitating fatigue due to anaemia
3. Decreased hospitalizations due to infections
4. Decreased need for platelet transfusions and/or red cell transfusions
5. Increased ability to perform normal activities of daily living
6. Increased quality of life: ability to continue working (many MDS patients must stop working or decrease the time they spend working); engage in social activities with their family, friends; resume their role within the family structure; engage in the physician activities (gardening, trekking, cycling, etc.) that they had the ability to do before they developed MDS

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.
- 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).

1. The bruising that occurs with subcutaneous administration of the drug.
2. Patients experience side effects including nausea and vomiting, fatigue for a period of time following the sequence of 7 days administration.
3. Visiting the hospital or clinic for 7 straight days.

Patients generally tolerate what they consider these ‘minor inconveniences’ well and tell us that they feel that this is a good trade off for the elimination of blood transfusions every week or two weeks, the increased level of functioning (with many patients returning to near normal life styles), that the side effects noted are easily controlled with proper administration of anti-nausea drugs and with proper administration of the drug.

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

Very few, if any patients, have told us (with more than 1,000 engaged in our Patient Quality of Life Forums worldwide) that they feel that this drug is not worth the small issues described above. The negative feelings we have heard come those patients who did not respond to the drug and feel that they now have no hope to improve or survive with MDS.

Some patients have side effects that are worse than others but these patients still feel the results are worth these side effects.

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?

This has not been determined although it is clear that MDS patients in the higher risk categories certainly have a much shorter life span due to the severity of the disease so it is obvious that these patients might be seen as benefiting MORE if they respond to treatment and their life span is increased.

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.

Transfusions (platelet and/or red cell), erythropoietin (if available in the patients postal code) are the standard of care for these patients. These are supportive care at best and do not influence the natural history of the disease.

They do not compare with treatment that does alter the natural history of the disease.

(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 in 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.)

1. Increased survival.
2. Decrease in or elimination of transfusions (red cell and/or platelets)
3. Decrease in infections and need for treatment for infection
4. Improved ability to function in all aspects of life
5. Improved ability to work
6. Reduction in the need to visit physicians, clinic, or hospital

(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).

1. Subcutaneous injections cannot be considered a disadvantage or difficulty if treatment eliminates the need for infusion of red cells, etc. and time spent in the hospital or clinic
2. Fewer trips are needed for treatment than are needed for the continuous care necessary with supportive care
3. Side effects are short lived and are manageable with proper drug administration etc.

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.

The experience in clinical studies are comparable what patients experience when VIDAZA is administered in the standard health care setting. Patients who receive this drug outside the clinical studies are not subjected to the mandated bone marrow aspirations and blood draws that are necessary in clinical trials so the experience is less invasive and tolerated better in the routine care setting of NHS.

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

No.

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.

The MDS Foundation has conducted more than 60 Patient and Family Forums worldwide. Many patients who attended these Forums have been treated with VIDAZA both inside and outside clinical trials. Our patient questionnaires and recorded discussions have yielded information reflecting an overwhelmingly positive attitude about this therapy and the hope that patients experience with this treatment. While this is not as yet published in a peer-review journal that publication is being readied for submission.

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?

1. Increased survival
2. Greatly improved quality of life
3. The ability to resume normal or near normal activities including work
4. Hope for the future

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

1. Loss of hope
2. Decreased life span
3. Loss of the ability to regain control over their own lives

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

This is variable. Patients either respond to therapy or fail therapy. It is not possible to predict the outcomes. Side effects are easily managed for the majority of patients.

Other Issues

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

Please do not consider these patients as a group of older patients (they are for the most part) who DO NOT deserve this therapy and deny them the chance to live better and longer lives.

Please do consider that this is the first hope that MDS patients have had that treatments will benefit them long term and that new therapies may ultimately remove the death sentence that is MDS.