

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]

Name of your organisation: Rarer Cancers Forum

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)

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.

- ***This condition can have a major impact on the quality of patient' lives. Fatigue may result in patients having to spend part of the day in bed and they may be unable to undertake everyday activities such as going for a walk, doing their shopping, working or enjoying social activities with family and friends.***
- ***The prognosis is poor and around 30% will progress to acute myeloid leukaemia***
- ***The technology gives them the opportunity to have an improved quality of life and longer survival***

(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.

- ***The technology alters the natural course of the disease by delaying or decreasing progression to acute myeloid leukaemia and improving survival***
- ***Research shows patients have improved quality of life, in particular in relation to fatigue, physical functioning, dyspnoea, positive affect and psychological distress compared with patients receiving only best supportive care (Silverman et al, 2002). Improvements such as these are enormously important to patients and their families.***
- ***Reduces need for blood transfusion with its risk of adverse effects (e.g. iron overload), inconvenience and discomfort***
- ***Technology is effective in all five subtypes of MDS***

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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).

- ***Not available orally - requires subcutaneous injections.***
- ***Myelosuppression is the most common side effect but in assessing this it is important to take into account the fact that the disease itself is associated with myelosuppression. Toxicity has been found to be a transient adverse effect and patients usually recover in time for their next treatment cycle (Silverman et al, 2002).***

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

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?

Comparing the technology with alternative available treatments or technologies

NICE is interested in your views on how the technology compares with with existing treatments for this condition in the UK.

(i) Please list any current standard practice (alternatives if any) used in the UK.

- **Best supportive care**
- **Chemotherapy**

(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.)

- **The comparators treat the symptoms**
- **The technology tackles the disease itself, improving bone marrow function through its probable epigenetic mode of action.**
- **It improves quality of life and survival.**
- **Subcutaneous injections can be administered in outpatients and for patients are more preferable than intramuscular injections, and vastly preferable to intravenous infusions.**

(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).

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?

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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.

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?

It would make an enormous difference, giving patients a chance to improve significantly both survival and quality of life

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

MDS is a very unpleasant disease with a poor prognosis. There's nothing else comparable with this technology so many patients would be condemned to an earlier death without the palliative effects offered by this technology. They would die knowing that the technology is available but denied to them on the NHS. They would feel totally let down.

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

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Other Issues

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

- ***The cost of the main comparator - best supportive care - should be very carefully considered e.g. not only is blood in limited supply, it involves a good deal of NHS staff time to administer.***