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?
- **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]
- 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.

The estimated life expectancy for sarcoma patients with metastatic disease is 8-12 months after receiving the first line of cytotoxic therapy. This technology offers the hope of long-lasting tumour control in a population of patients for whom the options have run out.

(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.

- ***Patients have a chance of living longer and being able to enjoy life for longer. This is very important in a group of patients who are frequently diagnosed late and are more likely to have advanced disease.***
- ***Hope is a key component of mental health – the feeling of having been given a fighting chance is very important for both patients and their families***

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).

- ***Central venous access is recommended as there may be severe injection site reactions if trabectedin is administered via a peripheral cannula***
- ***Neutropenia is common, but reversible and rarely associated with fever or infection (SPC)***
- ***Hepatobiliary disorders are fairly common but non-cumulative and reversible and IV dexamethasone administered prior to trabectedin appears to provide hepatoprotective effects (SPC).***

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?

Patients with raised bilirubin are unable to receive this technology.

Sarcoma subtypes – Schoffski, P.et al (2008) state that "Leiomyosarcomas and liposarcomas appear particularly sensitive to the drug. In myxoid and round-cell liposarcomas trabectedin seems exceptionally active". However, we would not wish sarcoma patients in other subgroups to be deprived of this drug simply because the numbers are too small and no trabectedin research has been done. That would be grossly unjust.

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.

There is only best supportive care – all components of this need to be carefully costed so that valid comparisons can be made with the cost of trabectedin

(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.)

As stated above, the technology offers the hope of living longer and enjoying life longer.

(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?

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?

Patients and their families would feel that the NHS had given them a last fighting chance to enjoy some extra time together – this is enormously important.

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

This is the first new drug for this condition for more than thirty years and patients and carers would feel extremely disappointed if this technology were not made available on the NHS. Patients would die knowing that they had been denied the chance of some extra time with their families and friends.

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

Other Issues

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

Criteria for appraisal of end of life treatments