

## 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] – on behalf of Trustees of BCRT

Name of your organisation:

**Bone Cancer Research Trust**

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) **Administrator replying on behalf of BCRT Trustees.**
- 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.

i) for the treatment of non-metastatic osteosarcoma: We expect the addition of mifamurtide to improve overall survival by about 10% as reported in Meyers et al Journal of Clinical Oncology 2008. This will have a important impact in a disease where there has been little improvement in outcome for 20 years in the UK.

ii) for the treatment of osteosarcoma with pulmonary metastases: We expect an improvement in 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.

We expect the major benefit to patients to be an improved chance of survival. The evidence points to a 30% reduction in mortality in the published randomised trial. We do not expect the technology to influence any of the other factors for patients.

The introduction of mifamurtide should have a significant benefit for patients' families and friends. The impact of death of a young person on family and friends is significant, often resulting in adverse short and long term health issues, particularly mental health. An improved survival for osteosarcoma should result in less short term and long term morbidity in family and friends.

**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).

We are unaware of any aspects that this technology might worsen.

We understand that the side effect profile of mifamurtide is relatively harmless and can be easily tolerated by most patients and side effects relieved simply.

We recognise that delivering mifamurtide will probably involve additional hospital visits for administration but the potential benefits mean that patients and families are willing to undergo the additional time burden.

There may be some additional financial impact on families having to attend for treatment but again this would be accepted by most patients and families given the benefits.

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

Not that we are aware.

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?

We understand that the published data is more supportive of the use of mifamurtide in patients with non-metastatic osteosarcoma than in those with metastases. We concur with this view but also think that mifamurtide should be available for all patients with osteosarcoma because the data indicates some benefit and we know that it will never be possible to conduct a randomised trial in this very poor prognosis group.

**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.

Current standard practice is combination treatment with chemotherapy (variations of cisplatin, doxorubicin, methotrexate, ifosfamide, etoposide) and surgery. Mifamurtide is given in addition to this standard therapy. There is no current standard alternative to mifamurtide.

(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.)

Improving survival – see above

(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).

See above

**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.

We are unaware of any patients who have received this technology routinely as part of NHS care

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

See above

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.

Not formal studies. We are aware that our supporters want to see the introduction of any new treatment that has strong evidence of improving survival.

**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?

As above. Improving survival is a major difference for both patients and carers.

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

Patients would not be able to access a treatment with demonstrable and long term benefit.

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

Not that we are aware.

**Other Issues**

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

Osteosarcoma is a rare disease (ultra –orphan) affecting mainly young people. A large randomised trial has been carried out demonstrating significant benefit in a disease where there has been little advance for over 20 years. We are aware that sometimes additional /confirmatory evidence is necessary but it is our view that a further trial is unlikely to be conducted and that even if it was, it would be more than 10 years until the results would be available. We would like this technology to be available as soon as feasible.