Appendix G – Patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Vemurafenib for the treatment of unresectable locally advanced or metastatic BRAFV600 mutation-positive malignant melanoma

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: Steve Chalk

Name of your organisation: Private Individual

Are you (tick all that apply):

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

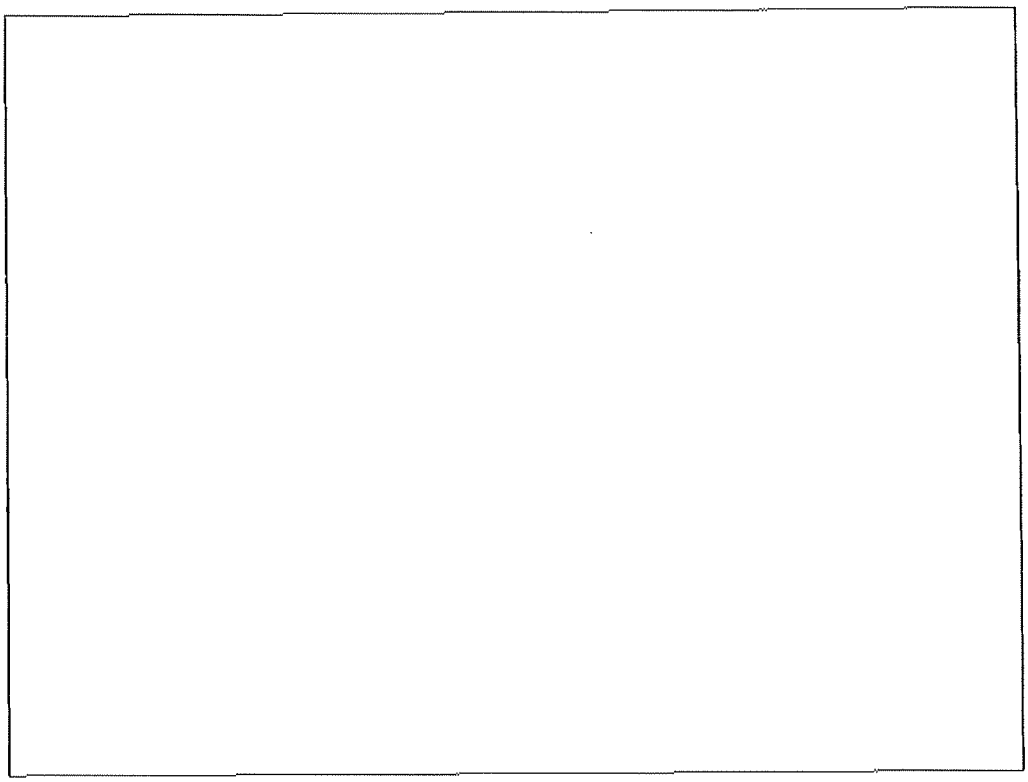
Currently diagnosed as a stage 4 melanoma patient with metastases to both lungs.

Appendix G – Patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Vemurafenib for the treatment of unresectable locally advanced or metastatic BRAFV600 mutation-positive malignant melanoma



Appendix G – Patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Vemurafenib for the treatment of unresectable locally advanced or metastatic BRAFV600 mutation-positive malignant melanoma

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 am expecting the Vemurafenib to halt the growth of my current tumours and to prevent spread of the Melanoma to other areas.

(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 benefits I have gained thus far are hard to quantify properly in words. In a matter of a few weeks I have gone from a situation of near hopelessness and resignation that my life was coming to an end, to one of optimism. The tumours on my lungs were clearly visible on X Rays in December but a month later appeared to have gone.

Aside from the obvious physical benefits, this has allowed me to continue on with life as normal. I am still working (thereby contributing to society as opposed to being a drain on resources) and my quality of life is exactly as it was pre-diagnosis. Mentally my overall quality of life has been lifted immeasurably. As an example I am now planning to run 100 miles over 3 days in June for charity – something I could not have contemplated a few months ago.

The effect has also been enormous on those nearest and dearest to me. The strain of a terminal diagnosis has been immense on my family and friends and my wife has suffered greatly. She has been off work as a result of stress but since my enrolment on the expanded trial for Vemurafenib and the benefits this has afforded me, she has recovered and is now back on an even keel, returned to work and has been discharged by her counsellor.

Appendix G – Patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Vemurafenib for the treatment of unresectable locally advanced or metastatic BRAFV600 mutation-positive malignant melanoma

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 thier family (for example cost of travel needed to access the technology, or the cost of paying a carer)

Thus far there have been no disadvantages concerned with the treatment. Side effects have been very minimal and not impacted on my ability to do anything. I have suffered a few aching joints but nothing for longer than a couple of days, an initial loss of appetite for a few days and a 'goosebump' type rash that has caused no issues as it is non-irrating.

Because the treatment is in tablet form it is easy to administer and Hospital visits are once a month for a couple of hours, which is very manageable and far less onerous than that required for other treatments.

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

As a member of a Melanoma forum I have been in contact with several people who are on this drug. Not one has had a bad word to say about it and I think it is fair to say that the 'Melanoma community' is genuinely excited by the results generated by Vemurafenib and the hope these have generated for sufferers.

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 don't believe one set of sufferers benefits less from the technology but my understanding is that if used early after metastases has been identified, the effects of the drug use are particularly successful and long lasting.

Appendix G – Patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Vemurafenib for the treatment of unresectable locally advanced or metastatic BRAFV600 mutation-positive malignant melanoma

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.

Currently treatment regimes for Melanoma in the UK are very archaic and basically unchanged for the past 30 years. Cut it out and hope appears to be the standard practise with any drugs used being for palliative use only.

(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 of 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)

Vemurafenib is a massive leap forward over current treatments available on the NHS. It works quickly and in many cases continues to work for a long time. It is very easy to administer as it is a tablet so patients can dose themselves at home and side effects (for me) have been miminal.

Slight joint pain in both wrists, right shoulder and left ankle. Only one site at a time and for no longer than 3 days per site. Best described as an ache as opposed to pain.

Loss of appetite and nausea from day 3 of treatment through to day 7.

Non-irrating rash which can best be described as permanent goosebumps.

Appendix G – Patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Vemurafenib for the treatment of unresectable locally advanced or metastatic BRAFV600 mutation-positive malignant melanoma

(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).

I am not currently aware of any disadvantage other than you have to have the correct gene to qualify for the treatment.

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.

Not known.

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

Not known

Appendix G – Patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Vemurafenib for the treatment of unresectable locally advanced or metastatic BRAFV600 mutation-positive malignant melanoma

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 known.

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 be a massive leap forward in the treatment of this terrible disease and would give hope where currently the NHS is unable to offer any.

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

With the results obtained through use of Vemurafenib so well known within the Melanoma community, it would be a devasting blow to sufferers and carers if the technology was denied to them by the NHS.

Appendix G – Patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal (STA)

Vemurafenib for the treatment of unresectable locally advanced or metastatic BRAFV600 mutation-positive malignant melanoma

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

None that I'm aware of.

Equality
Are there any issues that require special attention in light of NICE's duties to have due regard to the need to eliminate unlawful discrimination and promote equality and foster good relations between people with a characteristic protected by the equalities legislation and others?

None that I'm aware of.

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

No other issues.