NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal (STA)

Dabrafenib for treating unresectable, advanced or metastatic BRAFV600 mutation-positive melanoma [ID605]

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: Gillian Nuttall

Name of your organisation: Melanoma UK

Are you (tick all that apply):

- 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**) Founder and Key worker**
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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.

Improved prospect of quality, long term survival. There appears to be evidence of improved patient outcomes and experience to Melanoma UK's knowledge.

A highly innovative advance in treatment – One of the most innovate treatments in recent years.

This appears to be an effective therapy.

Targets an area of high unmet need. Melanoma is on the increase in the UK and leading clinicians have made it clear that there is a desire to be able to offer this treatment to all suitable patients.

(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.

Melanoma UK has anecdotal evidence from patients that we speak to and support that they have experienced astonishing results from this treatment. They feel that they have benefited to an enormous extent. We have reports of patients with such an improved quality of life that they have managed to return to normal life. This is echoed by families of patients. Without this treatment, they have no hope and mentally, this is tortuous for all concerned.

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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).

Whilst patients are aware that there might be certain side effects of the treatment, the patients that I have monitored and spoken to, are prepared to acknowledge the side effects and deal with them as and when they arise. The majority of patients that are supported by Melanoma UK are willing to tolerate the side effects of most treatments – in particular this one because they have seen other patients treated with it responding so well. It is worth noting here, that the alternatives for most patients are unthinkable. As far as any financial implications are concerned, most patients are willing to undertake treatments at a financial risk – given that should the treatment succeed, they might be in a position to return to full time employment in any event.

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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.

Not that I am aware of – but given what patients already know about this treatment, there is a strong appetite for its use.

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 is not a question that I am able to respond to with any accuracy – this would be easier answered by a medical expert.

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.

Dacarbazine
Ipilimumab
Vemurafenib (only for BRAF V600 mutation patients)

(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.)

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There is the issue of toxicity with use of dacarbazine – this can mean reduction in quality of life for some patients. This has been alleviated somewhat with the introduction of Yervoy in first line treatment. Both Yervoy and Vemurafenb have issues with side effects.

(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).

We have only spoken to a small number of patients who have been treated with Dabrafenib and overall we have not had any reports of serious adverse effects. We have no reports of worsening condition, but that does not mean to say that this is not an issue.

Equality and Diversity

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that this appraisal:

- Could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which [the treatment(s)] is/are/will be licensed;
- Could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;
- Could lead to recommendations that have any adverse impact on people with a particular disability or disabilities

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Please tell us what evidence should be obtained to enable the Committee to identify and consider such impacts

Nothing to add.

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.

NA

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.

NA

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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?

I will be bringing a patient expert to the Appraisal who is better placed to speak about this.

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

I can only give information provided to me from patients who have received this treatment and patients who wish to have this treatment should they need it in the future. They have expressed their utter desperation for such a treatment to be made available to them and respectfully urge the Committee to be pragmatic in their approach to this treatment.

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

Any patient with other illnesses such as leukaemia might be compromised but I could not say medically for certain what difficulties they might face.

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Other Issues

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

Until very recently, advanced melanoma patients have had little hope of long term survival. This new treatment is showing such promise and patients are reporting longer survival than anticipated. I speak regularly to a patient who was given an estimate of 6 months survival in 2012 and he is still alive. Melanoma is a brutal disease and patients are desperate for any treatments that might give them longer with their loved ones.