

**NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE**

**Single technology appraisal (STA)**

**Vemurafenib for the treatment of unresectable locally advanced or metastatic, BRAF<sup>V600</sup> 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: Gillian Nuttall**

**Name of your organisation: Factor 50**

**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) **I am the founder and CEO of a fundraising and patient support group.**

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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.

**Survival - I would expect this technology to improve the survival rate more than any other drug that is currently on the market. That has to be progress for melanoma patients.**

**Quality of life – once the side effects are dealt with there will be huge improvements to quality of life, compared to patients undergoing other unsuccessful treatments or with no treatment at all.**

**Mental stability – for patients who have survived the treatment and the illness, there will be the feelings of euphoria, compared to the despair that they were feeling previously. This will be a massive improvement for patients who do respond to treatment.**

**Quality of life for family and carers – at the end of treatment there will be distinct advantages in no longer having the worry and care of a sick relative to cope with.**

(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.

**If the patient is seeing a positive response to the treatment, their condition will be improving and the patient is seeing a change in their condition.**

**My understanding is that physical symptoms (say for example, actual tumours visible to the naked eye) are vastly reduced.**

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**There will be a vast improvement on the patients' mental health if they are responding positively to treatment.**

**If patients that are responding to treatment are able to function in a more or less normal way, following treatment, this is a very positive area to consider. Once a patient has responded to treatment and is back in work, this can only be a good thing for :**

- a. their own self esteem**
- b. their family**
- c. their work colleagues**
- d. society and the economy – when they are back at work they are continuing to contribute to the economy, taxes etc.**

**Another quality of life issue to consider is the fact that this disease is often a young person's disease. For parents not to have this treatment can leave young families devastated – young children without a parent, younger adults without partners, and in some cases, families who are losing children, still yet to contribute to the future.**

**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).

**I cannot say that I have any major concerns in relation to aspects of the condition that the technology cannot help with or may make worse. The majority of melanoma patients that I have spoken to, will try absolutely anything, irrespective of the side effects. I have discovered that there are patients who are prepared to tolerate anything at all. In my view, and in the view of the majority of patients, if there is to be a good outcome, the side effects are probably worth tolerating. The impact on family and friends etc, again, in the view of most patients, is worth tolerating for the good outcome.**

**I could see the possibility of travel being a disadvantage, but again, for a good outcome, a disadvantage that is tolerable.**

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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.

**All patients that I have had dealings with are very positive and hopeful about this technology. They hope that if they need it they will be in a position to get it.**

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 a question that I am unable to answer – there may be some medical answers to this, but if there are, I am not aware.**

**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.

**As far as I am aware, the current standard treatment is Dacarbazine – this is not an alternative – I understand that it has very low response rate and does not improve survival.**

(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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**My understanding is that a good outcome to this treatment is a great improvement in the condition.**

**Shrinking of tumours, and once the side effects are dealt with, a vast improvement in the mental health of patients and families.**

(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).

**If the patient is not treated the condition will become worse in any event, so I cannot see that the technology has any disadvantage. If the patient has standard or no treatment, the outcome will be poor.**

**As far as difficulty in use is concerned, the patients will be treated in hospital and under supervised medical conditions – they don't have to self administer any drugs, so apart from travel, no real disadvantage there. Most patients will not object to travel.**

**The side effects have been mentioned previously, and again, to reiterate, most patients will endure the side effects for the good outcome.**

**Research evidence on patient or carer views of the technology**

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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?

NA

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.

No

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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?

**It would mean the world of difference to patients and carers. At the moment, patients with this disease are faced with devastation, not only for themselves but for everyone around them. If this technology was made available on the NHS it would be the breakthrough that melanoma patients and doctors have been waiting for. It quite plainly may mean the difference between death and a prolonged life.**

**In my view it would also make a difference to society in general. Because this illness is a young person's illness and people do die at a very young age, the state is in fact losing out. We have educated people to good levels and then before they have the opportunity to contribute properly, we are losing them to this illness. The availability of the technology on the NHS can only be a positive outcome.**

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

**Simply that more people will die from this illness.**

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

**There are bound to be, for example patients with disabilities - but they would be cared for in a specialist centre with expertise of the medical team**

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**Other Issues**

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

**Apart from one other drug already evaluated, this is only the second treatment that is known to prolong the lives of melanoma patients. After patients and doctors being in somewhat of a wilderness for the last 30 or so years, we are now faced with what can only be seen as two breakthroughs in the treatment of advanced stage malignant melanoma. I would like the committee to consider this when appraising this latest technology.**