NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Patient/carer expert statement (STA)

(Melanoma (unresectable, metastatic) - pembrolizumab (after ipilimumab)) [ID760]

Thank you for agreeing to give us your views on this treatment that is being appraised by NICE and how it could be used in the NHS. Patients, carers and patient organisations can provide a unique perspective on conditions and their treatment that is not typically available from other sources. We are interested in hearing about:

- the experience of having the condition or caring for someone with the condition
- the experience of receiving NHS care for the condition
- the experience of having specific treatments for the condition
- the outcomes of treatment that are important to patients or carers (which might differ from those measured in clinical studies, including health-related quality of life)
- preferences for different treatments and how they are given
- expectations about the risks and benefits of the treatment.

We have already asked your nominating organisation to provide an organisation's view. We are asking you to give your views as an individual whether you are:

- a patient
- a carer (who may be voicing views for a patient who is unable to) or
- somebody who works or volunteers for a patient organisation.

To help you give your views, we have provided a questionnaire. You do not have to answer every question — the questions are there as prompts to guide you. The response area will expand as you type. The length of your response should not normally exceed 10 pages.

1. *About you*

Your name: [redacted]
Name of your nominating organisation: Melanoma UK
Do you know if your nominating organisation has submitted a statement?

☐ Yes ☑ No

Do you wish to agree with your nominating organisation's statement?

☐ Yes ☑ No

(We would encourage you to complete this form even if you agree with your nominating organisation's statement.)

Are you:

- a patient with the condition?

☑ Yes ☐ No

- a carer of a patient with the condition?

☐ Yes ☑ No

- a patient organisation employee or volunteer?
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☐ Yes ☑ No

Do you have experience of the treatment being appraised?

☑ Yes ☐ No

If you wrote the organisation submission and do not have anything to add, tick here ☐ (If you tick this box, the rest of this form will be deleted after submission.)

2. *Living with the condition*

What is your experience of living with the condition as a patient or carer?

I am a patient that has lived with metastatic melanoma since 2010. I originally had a 0.9mm mole on the upper left leg removed having noticed that it had altered slightly. I was informed following this removal that the mole had returned a pathology of malignant melanoma. Following this the next procedure was to carry out a wide local excision. This was completed with clear margins and so routine checks ensued at the local hospital.

In December 2012 I noticed a lump in my left groin. After a couple of weeks I raised this with the nurse at the hospital as it had not gone down. I was sent for a needle biopsy but this was clear. At MDT it was decided to remove the lump. This surgery took place in February 2013 and was declared positive for melanoma. I had an inguinal lymph node removal in late March and 5 nodes were found to be positive for melanoma.

As a result of this diagnosis and my local hospital offering little treatment options I asked for a second opinion at Southampton hospital. As a result of this I started a trial involving dabrafenib in combination with tramatenib. This was a year long program of treatment. At the end of this program I was classed as nil evidence of disease.

In December 2014 a scan showed that the melanoma had spread through the lymphatic system within the pelvis, right groin, abdomen and back. I had treatment with IPI. After two treatments the side effects from this treatment were not manageable and also a scan showed further progression.

I started Pembro on 5th March 2015 and to date have received 6 doses.

3. *Current practice in treating the condition*

Which treatment outcomes are important to you? (That is, what would you like treatment to achieve?) Which of these are most important? If possible, please explain why.

Currently there is no known cure for metastatic melanoma that cannot be operated on. As a patient the ultimate hope would be for a cure for this condition. It is understood that at present the data and information from pembro offers a shrinkage in the tumours within responding patients and therefore a prolonged lifespan and quality of life.

For me this is my aim is to have as long as possible without the disease affecting my quality of life.

What is your experience of currently available NHS care and of specific treatments? How acceptable are these treatments – which did you prefer and why?

In my experience there was little the local oncology centre would offer for advanced melanoma. I chose to attend a centre of excellence in order to be proactive about my treatment and options. This poses logistical issues with travel time and energy expenditure to this centre.

I have only one treatment option at present. If I had to compare it to other treatments an oral treatment would always be preferable however there is an acceptance that an infusion is my only option. The treatment is every three weeks and this is not too intensive nor does this adversely impact on my life plans to attend clinic for this treatment plan.

4. *What do you consider to be the advantages of the treatment being appraised?*

Benefits of a treatment might include its effect on:

- the course and/or outcome of the condition
- physical symptoms
- pain
- level of disability
- mental health
- quality of life (such as lifestyle and work)

- other people (for example, family, friends and employers)
- ease of use (for example, tablets rather than injection)
- where the treatment has to be used (for example, at home rather than in hospital)
- any other issues not listed above

Please list the benefits that you expect to gain from using the treatment being appraised.

Melanoma progression being slowed or reversed.

Pain from tumours being reduced

Quality of life improvement

Physical symptoms

Please explain any advantages that you think this treatment has over other NHS treatments in England.

Proven effect on patients in reducing tumours and therefore significant improving the quality of life of these patients and their families.

Although there are side effects for some patients these are not as apparent and frequent as with other drugs. Therefore the benefits the treatment can offer without the significant impact on quality of life is a real improvement on what is available.

If you know of any differences in opinion between you and other patients or carers about the benefits of the treatment being appraised, please tell us about them.

I am not aware of any differences of opinion

5. *What do you consider to be the disadvantages of the treatment being appraised?*

Disadvantages of a treatment might include:

- aspects of the condition that the treatment cannot help with or might make worse
- difficulties in taking or using the treatment (for example, injection rather than tablets)
- side effects (for example, type or number of problems, how often, for how long, how severe. Please describe which side effects patients might

be willing to accept or tolerate and which would be difficult to accept or tolerate)

- where the treatment has to be used (for example, in hospital rather than at home)
- impact on others (for example, family, friends and employers)
- financial impact on the patient and/or their family (for example, the cost of travel to hospital or paying a carer)
- any other issues not listed above

Please list any concerns you have about current NHS treatments in England.

I am not sure I have concerns overall, however not having pembro available as a first line treatment when it appears to be more beneficial than current options would be my current concern. I appreciate there is a cycle to all new treatments however there is a clear benefit to this treatment above any other available at the moment. In addition although there are side effects the majority of these are manageable without too much impact on activities of daily living.

Please list any concerns you have about the treatment being appraised.

I cannot think of any aspects the treatment cannot help with at this time. For me it has had a real physical and mental health benefit.

The treatment is infusion rather than tablet and so does require hospital attendance. This however is only every three weeks and therefore does not impact significantly on other activities. It is also worth noting that the infusion is short and therefore extended treatment sessions are avoided.

I have experienced side effects on this treatment, however these have been less than those on previous treatment regimes. These include fatigue/inability to sleep, palpitations, hand tremors, night sweats, joint pain and occasional upset bowel motions. The majority of these have been short lived or come and go. The main long term side effects have been fatigue and joint / muscle pain. This has continued through from the IPI and not really let up – I am therefore not able to say this is completely linked to pembro.

The side effects I accept as a given for the potential benefits the pembro may give. Despite the side effects I am still able to work in a full time capacity with some allowance from my employer in flexible working.

The fact there is a treatment option available gives family and friends help and hope for the future. The positivity this can bring to a situation cannot be underestimated. From my personal experience this far outweighs the side effects and also can allow family some sense of routine and belonging to the situation.

The financial impact of the treatment being in Southampton is a considered one. I could opt to alter my treatment centre however I am comfortable and happy with the centre I attend.

If you know of any differences in opinion between you and other patients or carers about the disadvantages of the treatment being appraised, please tell us about them.

Not that I am aware of.

6. *Patient population*

Do you think some patients might benefit more from the treatment than others? If so, please describe them and explain why.

All patients that qualify for the treatment and are responders. It would appear that with the successes this treatment has had this should be considered a first line treatment over first generation drugs such as IPI. IPI appears to have a lower success rate with greater side effects.

Do you think some patients might benefit less from the treatment than others? If so, please describe them and explain why.

Those where other less invasive options are available and considered to working.

7. *Research evidence on patient or carer views of the treatment*

Are you familiar with the published research literature for the treatment?

☐ Yes ☑ No

If you answered 'no', please skip the rest of section 7 and move on to section 8.

Please comment on whether your experience of using the treatment as part of routine NHS care reflects the experience of patients in the clinical trials.

Do you think the clinical trials have captured outcomes that are important to patients? Are you aware of any limitations in how the treatment has been assessed in clinical trials?

If the treatment being appraised is already available in the NHS, are there any side effects that were not apparent in the clinical trials but have emerged during routine NHS care?

Are you aware of any relevant research on patient or carer views of the condition or existing treatments?

☐ Yes ☑ No

If yes, please provide references to the relevant studies.

8. *Equality*

NICE is committed to promoting equality of opportunity and eliminating discrimination. Please let us know if you think that recommendations from this appraisal could have an adverse impact on any particular groups of people, who they are and why.

I am not aware of any conflict with any population group

9. *Other issues*

Do you consider the treatment to be innovative?

☑ Yes ☐ No

If yes, please explain what makes it significantly different from other treatments for the condition.

This is a second generation immunotherapy drug that has shown a greater success than previous. It is for all encompassing rather than other drugs that are specific to BRAF status. This drug has less side effects than previous options and that is a real advantage in maintaining the patients quality of life.

Is there anything else that you would like the Appraisal Committee to consider?

How important and life changing this drug is for so many patients suffering with melanoma. This is the first real time that a drug has been available that could significantly improve the outcome and quality of life for a significant part of the patient group.

10. Key messages

In no more than 5 bullet points, please summarise the key messages of your submission.

- Pembro has a much greater and proven success rate than other drugs before.
- The side effects can be managed with input from the oncology team and from my perspective they are managed well enough for me to continue working full time with a good quality of life
- Although invasive the regime is not overwhelming and allows for patients to function and have a quality of life around hospital visits.
- The implication for patients such as myself without access to pembro would be very bleak and would involve trials which impacts further on quality of life.
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