NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Patient/carer expert statement (STA)

[Insert long form title and ID number]

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: Katharine Robinson

Name of your nominating organisation: Lymphoma Association

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 ☐X No

- a carer of a patient with the condition?
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☐ Yes ☐X No

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

Do you have experience of the treatment being appraised?

☐ Yes ☐X 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?

Although I don't have Mantle cell lymphoma, I do have Follicular Lymphoma. Both of these are incurable and during the the period of diagnosis there was a month when I faced all of the NHL diseases (ie I considered the effects on me of all the various diseases), therefore I have a very good understanding of the psychological impact of facing this situation and of the effect of these diseases on family and friends.

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.

Obviously curing a disease like Mantle cell would be the gold standard but if one accepts that this is currently impossible, then achieving the longest and most side-effect free period during the first remission is the most important thing. However I would be prepared to accept a level of side effects in order to gain a longer period of treatment free life. Also the longer time that is achieved the greater the chance that other new drugs will have come on stream therefore increasing lifespan.

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

I am personally on "watch and wait" which is the situation for a small number of mantle cell lymphoma patients. Like them I am constantly looking at my

options and to see a treatment like Bortezomib approved would give a great boost.

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.

The length of time in first remission being greater than current treatments. This would also help with the mental health of the patient but also for those close to them. Giving the treament sub-cutaneously would also be helpful in coping with the treatment.

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

Length of time to next treatment as I have stated above. A large number of these patients are men who will just have retired. To get three to four years of more normal life might enable their grandchildren to remember the. For the patient and their partners to have a couple of years retirement at the end of their lives in relatively good health is invaluable.

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.

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.

At the moment the current treatments for mantle cell just don't work for very long and also have side-effects. The knowledge of this must be hard to bear.

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

I think the issue of side-effects would be a concern however they do seem to mainly during the treament period and a longer length of period without treatment is something that I think most patients would be prepared to risk extra side-effects for.

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.

6. *Patient population*

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

For a patient that has good performance status but for some reason cannot have or refuses transplant (say on religious grounds) this might be a very important option.

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

Weaker patients may not be able to use this treatment but this is true of the current drug regime too.

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

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

☐ Yes ☐x 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 □x 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.

This treatment might be an option for stronger patients who refuse transplant on religious grounds. This would therefore have an equalising effect for a small number of people.

9. *Other issues*

Do you consider the treatment to be innovative?

□x Yes □ No

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

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

Do not spend fast amounts fo time considering whether this drug can increase overall survival as this is really unknowable at present and irrelevant to mantle cell patients who are likely to die wthin five years with current regimes. What is imortant is to give them more time now and by doing so you may bring them in a period of time that might see even better treatments. It would certainly give them a chance of number of years of more normal life.

10. *Key messages*

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

- In this disease, until a cure is found, it is essential to concentrate on making the first remission has long and as normal as possible.

- This is an aggressive lymphoma that currently does not have good results this drug could give a chance to survive with the hope of other drugs coming online the next few years, but in the meantime an extra couple of years would be very significant.
- This would give hope to mantle cell patients, their families and friends.
- Other drugs maybe on the horizon but for someone with a terminal disease it is the here and now that counts.
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