NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Patient/carer organisation submission (STA)

Bortezomib for previously untreated mantle cell lymphoma [ID724]

Bortezomib for previously untreated mantle cell lymphoma [ID724Thank 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, and including health-related quality of life)
- the acceptability of different treatments and how they are given
- expectations about the risks and benefits of the treatment.

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 length of your response should not normally exceed 10 pages.

1. *About you and your organisation*

Your name XXXXXXXXXXX

Name of your organisation: Leukaemia CARE

Your position in the organisation: [redacted]

Brief description of the organisation:

Leukaemia CARE is a national charity; founded in 1967 and first registered with the Charity Commission in 1969; which exists to provide vital support services to patients, their families and carers during the difficult journey through the diagnosis and treatment of all forms of blood cancer (leukaemia, lymphoma; Hodgkin lymphoma; non-Hodgkin lymphoma; multiple myeloma; myelodysplastic syndrome; myeloproliferative disorders & aplastic anaemia).

Our current membership database stands at approximately 13,500 (this includes patients, carers and members of the patients immediate family members.)

Leukaemia CARE offers this support through its head office, based in Worcester and a network of volunteers all around the United Kingdom. Support is offered over seven key areas:

- 24-hour CARE Line and live chat (currently office hours only)
- Support groups
- Patient and carer conferences
- Nurse conferences
- One-to-one phone buddy support
- Cancer campaigning and patient advocacy
- Information and booklets (Information Standard Approved.)

Since its inception over 25 years ago our CARE-Line has taken many thousands of calls from patients, their carers, family and friends. Our website provides extensive information on all aspects of the blood cancer journey, running from diagnosis to what happens when treatment stops and includes information on the emotional impact of a blood cancer and help for those caring for a patient. Our focus is purely on supporting anybody affected by a

diagnosis of blood cancer, simply supporting a quality of life for all (see - http://www.leukaemiacare.org.uk)

Leukaemia CARE also works with other charities and policy/decision makers to campaign for the rights of all patients affected by a cancer of the blood to have access to and receive the best possible treatment and care when they need it.

Organisational Funding:

Over 90% of our total funding come from our own fund raising activities, either via our members and fund raisers, legacies, grants, on-line shop, Christmas card sales, recycling exercises etc.

Leukaemia CARE receives funds from a wide range of Pharmaceutical companies, but in total those funds on average do not exceed more than 10% of our total income. The funds received from the Pharmaceutical Industry are received and dispersed strictly within the Guidelines as laid down by the ABPI Code of Practice 2015, Clause 27 - Relationships with Patient Organisations.[1]

We also operate strictly within the Guidelines defined by the "Leukaemia CARE Code of Practice."[2] This Code of Practice governing corporate funding is a commitment undertaken by Leukaemia CARE regarding our financial relationships with all commercial entities and the pharmaceutical industry in particular. Both of these documents can be examined via the hyperlinks listed below, or they are available in hard copy upon request.

We pride ourselves on our independence from any external influence/undue pressure arising from any of the other stakeholder bodies operating within the same sphere of activity as ourselves – the Industry, the NHS, the DoH, NICE, the Medical Profession etc., all bodies that we work closely with but are independent from. We will maintain our independence to the best of our ability and eschew any support that could adversely impact our reputation. This fact is made clear to any drug company (or other body) seeking our advice/assistance at the time of first contact. Our Code of Practice is also shared with them at that time.

1 - http://www.pmcpa.org.uk/thecode/InteractiveCode2015/Pages/clause27.aspx
2 - http://www.leukaemiacare.org.uk/code-of-practice

(For example: who funds the organisation? How many members does the organisation have?)

We are asking for your collective view as an organisation and will be asking patient experts for their individual input separately. If you have the condition, or care for someone with the condition, you may wish to complete a patient expert questionnaire to give your individual views as well.

2. Living with the condition

What is it like to live with the condition or what do carers experience when caring for someone with the condition?

Mantle cell lymphoma (MCL) is a rare form of non-Hodgkin lymphoma (NHL), which mainly affects males over the age of 60. Approximately 5% of people diagnosed with NHL have MCL.

As with other forms of NHL, MCL is a cancer of the lymphatic system. MCL is a quickly developing 'high-grade' form of NHL, that if left untreated grows quickly.

The most common sign of MCL is a "rubbery" lump in any/some/all of the neck, armpit, groin or stomach. Other symptoms include frequent and persistent infections; fever; drenching night sweats; severe fatigue; itching; unexplained weight loss and pain in the chest, abdomen or bones. These symptoms can be extremely draining and hugely impact on quality of life.

3. Current practice in treating the condition

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

The key aims of current treatments are to increase survival times and induce long-lasting remissions for patients. In addition to this patients would like to see reduced side effects and improved quality of life.

However, there have been numerous trade-off studies which show that many patients are willing to endure increased side effects if the treatment has improved efficacy.

What is your organisation's experience of currently available NHS care and of specific treatments for the condition? How acceptable are these treatments and which are preferred and why?

Treatments for MCL vary greatly depending upon the stage of MCL. A small number of patients will not need to start treatment straight away which is known as 'active monitoring' or 'watch and wait'. However, almost all patients will start treatment soon after diagnosis.

The main ways in which MCL is treated are:

- Chemotherapy - Cell-killing drugs - Steroids are normally used along with chemotherapy
- Radiation therapy - Use of x-rays or other radiation to kill lymphoma
- Targeted therapy - Drugs which recognise and kill lymphoma cells
- Biological therapy - Treatments which use the immune system to destroy lymphoma cells
- Stem cell transplant - fitter patients may be given a stem cell transplant (bone marrow transplant).

MCL is difficult to cure and tends to relapse quite quickly after treatment, so patients will usually need more than one type of treatment.

The efficacy and side effects of these treatments varies greatly. However, overall there remains a clear need for a treatment that can extend survival and induce prolonged responses.

4. What do patients or carers 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 patients or carers expect to gain from using the treatment being appraised.

The key benefits patients expect to gain are:

- Extended progression-free survival
- Improved response rates
- Increased duration of response
- Extended treatment-free interval

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

See above.

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

N/A

5. What do patients and/or carers 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 patients or carers have about current NHS treatments in England.

Current treatment options vary greatly. However, some common concerns include: the risks of stem cell transplants, relapsing, the duration of response to treatment etc.

It is important to note that the limited efficacy of many current treatments can hugely impact on the psychological (and consequently physical) wellbeing of patients. Access to more effective treatments reduces fear/worry and consequently improves the quality of life of patients (as well as their carers, family and friends).

Please list any concerns patients or carers have about the treatment being appraised.

The available trial data appears to show that bortezomib has an increased side effect profile. As such, the group of patients who are able to tolerate the treatment may be limited. However, for the patients who can it may have improved efficacy.

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

N/A

6. Patient population

Are there any groups of patients who might benefit more from the treatment than others? If so, please describe them and explain why.

The patients who stand to benefit most would be those who are fit enough to tolerate the increased side effects.

Are there any groups of patients who might benefit less from the treatment than others? If so, please describe them and explain why.

The patients who would benefit less than others would be those who are not fit enough to withstand the increased side effects.

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

Is your organisation 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 patients' experience of using the treatment as part of their routine NHS care reflects the experiences 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 (for example, qualitative studies, surveys and polls)?

☐ Yes ☐ No

If yes, please provide references to the relevant studies.

8. Equality

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular

protected characteristics and others. Protected characteristics are: age; being or becoming a transsexual person; being married or in a civil partnership; being pregnant or having a child; disability; race including colour, nationality, ethnic or national origin; religion, belief or lack of religion/belief; sex; sexual orientation.

Please let us know if you think that recommendations from this appraisal could have an adverse impact on any particular groups of people, such as:

- excluding from full consideration any people protected by the equality legislation who fall within the patient population for which the treatment is/will be licensed;
- having 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 treatment;
- any adverse impact on people with a particular disability or disabilities.

Please let us know if you think that there are any potential equality issues that should be considered in this appraisal.

Men are more than twice as likely to develop MCL (than women).

Are there groups of patients who would have difficulties using the treatment or currently available treatments? Please tell us what evidence you think would help the Committee to identify and consider such impacts.

Any patients who are less fit and consequently unable to tolerate the increased side effects will have difficulty using this treatment.

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.

Are there any other issues that you would like the Appraisal Committee to consider?

N/A

10. Key messages

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

- Mantle cell lymphoma is a rare type of non-Hodgkin lymphoma, with a range of debilitating symptoms that can hugely impact on quality of life.
- There are currently limited treatment options for patients. As such there is a clear unmet need for effective treatment options.
- The data appears to show that bortezomib increases progression-free survival and increased the duration and rate of response.
- Bortezomib has an increased side effect profile and as such some patients may not be able to tolerate this treatment.
- A diagnosis of blood cancer has a ripple effect, not only affecting the patient but impacting upon their carers, family and friends. As such improvements in treatment options benefit not only patients, but their carers, family and friends as well.