

Patient/carer organisation statement template

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: [REDACTED]

Name of your organisation: **Chronic Lymphocytic Leukaemia Support Association CLLSA**

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) [REDACTED]

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.

Broadly speaking at present the CLL patient population is divided into what has been described as 'go-go', 'slow-go' and 'no-go' groups. This describes the whole range of the fitness of the population. Fit patients, with few or no co-morbidities, (GO Go) can be treated with the combination chemotherapy and monoclonal antibody treatment Fludarabine, Cyclophosphamide and Rituximab, (FCR) approved by NICE.

Less fit patients, those who are not well enough to tolerate FCR (Slow Go) are assigned to chlorambucil, a gentler, less effective treatment.

Patients who have many co morbidities and those who have had many treatments and who will not respond to treatment are assigned to supportive care.

This description is very superficial, for many reasons; CLL is not a straightforward disease and patients may develop clones of the disease that are resistant to various agents. Even with the initial treatment some sub groups of CLL may not respond to fludarabine based treatments. Less fit patients may fail chlorambucil treatment and the patient and physician may then cautiously use FCR, which is not ideal. The indications are that Bendamustine even as monotherapy would be both effective and suitable for some patients who are not deemed fit for FCR treatment, improving the treatment of this group of 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

The evidence for bendamustine, especially anecdotal evidence, is that CLL patients derive a significant advantage from the use of Bendamustine. Used in situations where Fludarabine based treatments have failed to work, many patients have been delighted to get relief from the disease and some degree of remission. Statistical evidence shows that Bendamustine is a more effective treatment than chlorambucil, giving more relief from disease and longer remissions. Time in remission is extremely valuable to patients. Also, the greater the time between the need to treat relieves some workload from the NHS

Appendix I – Patient/carer organisation statement template

- Physical symptoms

- pain

Physical symptoms connected to bulky glands will be relieved; less pain, discomfort, better ease of movement when lymph glands have impaired joint function. Night sweats will not occur; as the patient recovers from the treatment, fatigue should reduce. Muscle cramps will be less frequent.

Some immunological problems such as abnormal sensitivity to insect bites will resolve. In time (the immune system has to recover) the patient should be less susceptible to infections, though at this stage of CLL the immune system is damaged.

- Level of disability

Level of disability will reduce as the patient recovers from treatment. Some benefits include reduction of fatigue and node bulk; relative freedom from infection can take longer, as the immune system recovers from treatment. As treatment continues fatigue can return due to the toxicity of the drug, but may alleviate with time.

mental health

Mental health is linked to the patient's degree of independence, and their hope of a remission. As the patients notes their increase in general health and is told of the improvement in blood counts, they become less anxious. As independence increases, mental health will increase; the patient perceives that they are more able to contribute to the family, socially, and often in a work situation

- quality of life (lifestyle, work, social functioning etc.)

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Quality of life; anecdotally QOL during a remission is much greater than that of a patient who feels ill and believes that chemotherapy will be necessary in the near future.

Other quality of life issues not listed above

Other people (for example family, friends, employers)

Ability to work; some patients are able to work and long remissions will enhance this. This benefits employers since they are retaining an experienced and well trained employee.

Social functioning; patients are able to rejoin social groups such as hobby groups, gym, dancing and religious meetings. This is immensely valuable for the patient.

Any increase in the patient's health and ability to contribute, to work and to follow interests will have a positive benefit for the patient's family. It will also decrease the time which the family has to spend caring for the patient.

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

All chemotherapy has a detrimental effect on the immune system of the patient and their ability to avoid or heal infections. Bendamustine is no different in the adverse effects occur during and after treatment, though it is described as well tolerated.

- Difficulties in taking or using the technology

Bendamustine is given in a hospital environment by injection. I have asked several CLL patients if they feel that the extra hospital visits and discomfort of this form of treatment would put them off using Bendamustine given the possible advantages of the drug, and the answer has resounding been that they would willingly tolerate the difficulties.

- Side effects (please describe which side effects patients might be willing to accept or tolerate and which would be difficult to accept or tolerate)

Patients are individual when they think of side effects, but generally CLL patients will tolerate the usual chemotherapy side effects (nausea, vomiting, hair loss should it occur, possibility of immune system damage,) if on balance they have a chance of relief from the disease. But, as ever, patients do need to be informed of the possible side effects and the chances of developing the effects, and how long they may last.

- impact on others (for example family, friends, employers)

In the short term, patients under treatment need more care from carers and may be unable to work or have their work hours reduced. Long term there should be an improvement meaning less care.

- 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).

Patients for CLL fall under the 'watch and wait' regimes. Treatment will be given when necessary, so that these costs must be met at that time. I have not heard of patients refusing any treatment because of lack of funds for travel, but in some communities travel may cause difficulties.

Appendix I – Patient/carer organisation statement template

3. Are there differences in opinion between patients about the usefulness or otherwise of this technology? If so, please describe them.

I have not come across any differences of opinion of the usefulness of bendamustine and I have had unsolicited testimonials to its effectiveness.

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?

Please see the rough patient groupings given above. The physician would allocate the appropriate treatment to each patient from the patient's needs and level of fitness. On present information, if a patient is fit enough to receive FCR and FCR is effective, then FCR would be preferable since there would be a likelihood of a longer remission and an extension of life.

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.

Chlorambucil- often used in patients with significant co-morbidities or kidney problems. Chlorambucil- is used for First and second line (subsequent) treatments of less fit patients.

Fludarabine, Cyclophosphamide, Rituximab-(FCR) standard first line treatment for fit patients. Before the introduction of FCR for first line by NICE, FC was used. . FCR second line is adopted by NICE under certain conditions. .

Campath –Alemtuzumab (for TP 53 deleted cases, and when FCR proves an ineffective treatment.)

CHOP, often R-CHOP as CHOP is also used when the CLL does not respond to FCR or some other combinations.

Transplant- mini-allo, matched unrelated donor (mud) transplants have a good success rate, followed by sibling matched donors. Transplant is preceded by intensive conditioning. Transplant is generally considered a salvage treatment, but some centres consider it appropriate for fit patients at first relapse.

Steroids are often used with any of the above to drop the white cell count or in cases of AIHA.

Use of all of the above is dependant on the relative health of the patient. In general, a drug is less effective the second time it is used, though if the remission is ≥ 2 years the physician may well decide to repeat the use of the original treatment, depending on cell markers.

Appendix I – Patient/carer organisation statement template

(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

Bendamustine shows an advantage for patients in the less fit group, over the chlorambucil treatment now available.

- Improvement in certain aspects of the condition

The more drug combinations that are made available to the NHS, for effective treatment CLL, the longer that the CLL patient can live with a good QOL.

Addition of bendamustine to the drug list would give a tolerable drug that would be available when fludarabine based treatment is ineffective or undesirable. At present FCR is the only effective combination to show increased time in remission and extension of life, the indications are bendamustine certainly adds to the increased time in remission.

- 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.)

All chemotherapy drugs have side effects- see below.

(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)

Bendamustine is administered in hospital rather than chlorambucil tablets at home.

- where the technology has to be used (for example in hospital rather than at home)

See above.

- side effects (for example nature or number of problems, how often, for how long, how severe).

There is no firm evidence at present that the side effects are not commensurate with the effectiveness of the drug; this is chemotherapy and there are toxic effects associated with it.

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.

I have had favourable comments from patients who have received bendamustine, but not as first line treatment. In most cases, bendamustine was extremely effective, in another case partially effective.

Are there any adverse effects that were not apparent in the clinical trials but have come to light since, during routine NHS care?

Not to my knowledge.

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.

Not directly, but I am aware of QOL studies.

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?

Patients; greater QOL overall, due to better treatment and longer remissions. Carers; the relief of knowing that the person they are caring for is experiencing enhanced QOL. The carers' burden will be reduced.

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

For both patients and carers, the situation would not change from the present. There are few effective treatments available for CLL, and the disease always recurs if the patient survives. So effectively, if Bendamustine is not added to the drugs available, the overall QOL of CLL patients will not improve.

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

Those patients who do not want to be treated by injection or infusion.

Other Issues

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

None