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

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? Chairperson CLLSA. ✓

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.

Patients receiving Rituximab as a part of their treatment have been shown to have a longer time in remission.
Patients can achieve more 'complete remissions' with the FCR protocol than with FC. Absolute tumour load becomes lower by modern standards. Response rates are higher.

It is feasible that since patients given FCR experience longer remissions, it may extend their life expectancy. This is difficult to measure given the relative brevity of trial follow up and the relatively long mean survival time 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

See (a) above. The longer that a patient stays in remission, the better their relative health and overall quality of life.

- physical symptoms

The physical symptoms of CLL are greatly varied from patient to patient; some experience fatigue, night sweats, weight loss and are susceptible to infections. Since any treatment increases the likelihood of temporarily damaging the immune system, the susceptibility of patients to infections will continue for a time after treatment. However, the greater the interval between treatments, which FCR increases, the less time overall the patient will be susceptible to infections. The post treatment problems with FCR are similar to other cancer treatments in general, that patient is often fatigued for a time. Patients with FCR will have lower T cell levels for up to 2 years, which will make them susceptible to infections. Patients on FCR are routinely supported with prophylactic antibiotics, and monitored for CMV reactivation. This is not dissimilar to the present FC regime.

- pain

Patients have told me that they experience pain when nodes are enlarged. FCR shrinks nodes as the disease is controlled. Similarly, patients can experience what is described as 'discomfort' if the spleen enlarges beyond a certain stage; lowering the burden of the disease may rectify this, but there are other choices a medic may use. Some patients have described muscle cramps as the CLL progresses that go away with treatment. There is no reason to suppose treatment with FCR would not do this. Advanced patients can experience pain as the bone marrow becomes filled with leukaemia cells.

- level of disability

Overall and over time the increased remission time should decrease the level of personal disability.

- mental health

Mental health in the CLL patient depends to some degree on their understanding of their condition, and certainly their co-morbidities. For the more informed patient, the knowledge that there is a more effective treatment available, even if it is not a cure, extending the remission time, would give comfort and hope, improving mental health.

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

With the longer remission time, better response rates, the lifestyle issues with CLL would probably be resolved for longer than with other treatments. The compromises which fatigue and susceptibility to infection demand would be minimised for a greater period of time. Many patients are concerned with becoming infected with viruses and bacteria that are rife among the general population especially in winter, and are also worried about contact with young children who may have had immunisations, limiting time with their own relatives. .

- other quality of life issues not listed above

I'm convinced most carers will be relieved at a better treatment protocol, it will take much of the burden off them for a while.

- other people (for example family, friends, employers)
- *Again, friends and family will respond favourably to an extended remission. Employers will as well; it means that their investment in senior employees will be less likely to be terminated before natural retirement.*
- other issues not listed above.
- *What the better treatment offers is definitely hope; the field of CLL treatment is advancing extremely fast, and the longer that remissions last the more hope there is that the next treatment will be even more effective, or even a cure.*

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.

Only a proportion of patients will be suitable for the FCR protocol. Patients may be excluded due to co-morbidities or lack of a functioning P53 pathway. Medics can distinguish these groups and the technology need not be applied unwisely.
For the patients who are suitable for FCR, there may be long term side effects due to Rituximab in a small number of cases. Some patients cannot be treated with Rituximab more than once as they develop sensitivity to the 'mouse fragment'. A few patients develop delayed neutropenia.

- difficulties in taking or using the technology

Rituximab is given as IV or SC; the preparation must be done by the pharmacists, and given generally under day patient conditions. It is not suitable for self administration.

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

First infusion is accompanied by the 'shake and bake' or tumour lysis syndrome, another reason why this drug is not suitable for self administration. Patients are given the appropriate supportive treatment. I understand that this seldom occurs more than once byt patients must be monitored. I am not aware of any patients who have ceased treatment because of this. I am told that it is minor compared to the effects of FC. Rituximab is described as being well tolerated by patients in multiple injection treatments.

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

FCR requires day patient attendance, necessitating time for attendance and travel. Some other chemotherapy regimes are tablet only, and can be self administered at home without supervision.

- 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).

- *This is very dependant on where the patient lives compared with the place of treatment. Most patients who are well enough to receive the FCR would have the ability to travel. Extremely elderly and infirm patients would not be receiving FCR. It is undoubtedly true that hospital treatment will incur greater personal travel costs than home administration*.

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

I put this question to a recent meeting of the patient group, and they were unanimous and vocal in their support for the supposition that FCR should be a part of the UK NHS medic's armoury of treatments for CLL. There were no dissenters.

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?
See 'disadvantages' above.

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.

The most commonly available treatments are the following;

Chlorambucil;

Fludarabine and cyclophosphamide (FC)

CHOP (cyclophosphamide, doxyrubicin, vincristin and prednisolone;).

Alemtuzumab, called MabCampath, Campath with 'High Dose' prednisolone, 'High Dose Steroids' (HDS).

Bone Marrow Transplant

(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.)

Rituximab containing therapies offer an improvement on the condition of the patient overall.

The increased time between treatments is proven and this may lead to further advantages that medium term studies could reveal, i.e., increased survival time, more limited progression of the disease. Response rates are higher with rituximab combination therapy.

(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).

Disadvantages.
Rituximab has to be administered by injection which entails the drug being prepared by the hospital pharmacy department and administered under medical supervision. This is a disadvantage over the more conventional chlorambucil and FC treatments, but comparable to CHOP, conditioning treatments and BMT. Rituximab is less costly that BMT from the hospitalization point of view.

Side effects would be additive to present therapies, but are more acceptable to patients than the side effects of FC, CHOP, or BMT; Patients consider that the addition of Rituximab to FC is not a reason for rejecting the treatment.

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.

The patients to whom I have spoken who have used rituximab have been satisfied with the results.

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. The reported incidents of late onset neutropenia and 'allergy' to the mouse fragment have come form reports from USA patients.

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.

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?

Availability of Rituximab would make a large difference to patients and careers. Both would be confident that longer remissions were likely, which would in turn relive a great deal of stress on both the patient and the carer.

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

The informed patients are extremely discontented with the perceived inadequacy of cancer care in general in the UK, and particularly as it applies to their own condition, CLL. The knowledge that this is technology that has been widely available throughout the civilised world for many years, but is not available to UK NHS patients, is galling and to many, inexplicable.

There is also the underlying current of ageism, in that patients have complained to me of the stark choice that they have had in 'buying' extra time at the end of their lives, at the expense of their family, and their despair at being 'let down' by a system into which they have paid for 4+ decades.

Denying Rituximab to CLL patients would reinforce both of these views.

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

Rural patients due to travelling problems.

Other Issues

Please include here any other issues you would like the Appraisal Committee to consider when appraising this technology.
None.