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: Jacquelyn Williams Durkin

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?**X**
- 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) **X Trustee**
- other? (please specify

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.

I *would expect the technology (Rituximab) to reduce the level of leukaemic B cells in the blood, and specifically to reduce the size of lymph nodes. There will also be a reduction in the so called 'b cell' symptoms, eg sweating profusely, tiredness.*

At the end of treatment, the elimination of the cancerous B cells enables the production of healthy B cells which results in the opportunity for a functioning immune system. When speaking with CLL patients and their carers, the constant nature of repeated infections, sweating and tiredness becomes increasingly insidious and impacts directly on quality of life.

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
- physical symptoms
- pain
- level of disability
- mental health
- quality of life (lifestyle, work, social functioning etc.)
- other quality of life issues not listed above
- other people (for example family, friends, employers)
- other issues not listed above.

Short term benefits: would include response to technology that may result in quick reduction in lymph node, (or other impacted organ such as the spleen) size.

Long term benefits: CLL patients look for outcomes that extend time to next relapse and need for further treatment. In combination with other chemotherapy drugs this technology provides an opportunity to impact positively on the amount of time a patient remains in remission.

The longer someone stays in remission the more the overall level of health and well being improves. A longer duration of remission enables a person to experience a real and tangible period of feeling well and functioning normally. This means being able to undertake tasks, journeys and work on consecutive days without needing a 'day to recover'. It would also enable planning of holidays (and access to health insurance cover) and looking forward to future events eg wedding anniversaries. The world of the CLL patient becomes very small; every action, trip, event has to be planned in order to minimise exhaustion. If the body has a longer period of wellness and recovery then the patient is better placed for the next round of treatment.

The impact on family and close carers is profound as their lives also become dependent on the ability of the sick member to function in a given situation.

A paper in late 2008, written by Dr Claire Dearden and published in the journal 'Blood' details these complications. ' CLL is a disease characterised by a poor immune system, which results in an increased susceptibility to infection, particularly respiratory infections. In addition complications such as autoimmune disease are also not uncommon, with reduced immunoglobulin levels even in early stage disease. faced by those diagnosed with CLL'. This paper also indicated that monoclonal antibodies (including this technology) show promising results in preventing and managing CLL associated autoimmunity.

Therefore the impact of CLL on the health of patients can be profound with or without treatment.

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.
- difficulties in taking or using the technology
- side effects (please describe which side effects patients might be willing to accept or tolerate and which would be difficult to accept or tolerate)
- impact on others (for example family, friends, employers)
- 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).

Side effects of technology: the main drawback is a possible initial adverse reaction to the infusion – shivering and high temperature – a type of allergic reaction. This is a well documented effect and staff in chemotherapy units should be well versed in the appropriate actions to take – use of antihistamine and steroids, and reducing the rate of infusion. Some individuals may never be able to tolerate the technology but my understanding is that for most patients further infusions are well tolerated.

If this technology is added to other chemotherapy that is usually taken at home in tablet form (Fludarabine and Cyclophosphamide), then a trip to hospital/or chemotherapy centre for infusion will be necessary. This additional trip may not be welcomed by patients. Hospitals will need to ensure there are enough beds in day units to accommodate extra patients.. The introduction of delivering chemotherapy infusions at home by private providers is being looked at by Cancer Networks although Mabs do present particular infusion difficulties.

As with all chemotherapy, there is a risk of side-effects – neutropenia, leading to risk of severe infection, thrombocytopenia (blood clotting problems) and red blood cell production leading to anaemia

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

Most opinion expressed has related to whether the technology does in fact improve the efficacy of other drugs. At patient conferences this year (09) there was a great deal of interest in recent research that seems to report that the technology adds to the impact of existing chemotherapy thus extending length of remission.

There is a difference in perception in those who have undergone chemotherapy for CLL and those in 'watch and wait'. Those who have had chemotherapy already (and perhaps had more than one course of treatment) are concerned about toxicity and side-effects of additional technologies being added to existing drugs. However the

advantages of longer remission time overshadow these concerns. There is also the hope that newer treatments may come on stream in the future.

Some patients are aware that in the USA the technology is used as a sole maintenance drug to keep CLL under control. They are also aware that it is possible to become refractory to the technology ie the technology stops being effective

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?

Our understanding is that the technology is better at clearing out the CLL from lymph nodes than in the bone marrow or peripheral blood. It may be that a subset of CLL patients responds better to the technology than others. This would require cytogenetic testing for specific abnormalities and access to trial data..

A paper in the British Journal of Haematology by Keating et al (BJH 141 Apr 2008) suggested that CLL patients with a chromosomal abnormality - trisomy 12 -showed a larger number of CD20 antigenetic sites than patients with other abnormalities. CD20 is targeted by the technology. These patients showed a high response rate when given the technology.

However the 'REACH' trial (results presented at ASH in Dec 2008 - see 50th ASH *online program and abstracts) showed that many patients in the trial, with various cytogenetic abnormalities and at different stages of disease responded well to this technology. So it would appear that all patients with CLL can benefit from this technology*

Patients that do less well with the technology are those who have co-morbidities ie with other health impairments, for example kidney problems, that can affect their ability to cope with the side effects of the technology. This may lead to periods of hospitalisation; however this is the case with other technologies and the REACH trial seemed to show only a slightly increased risk of what are known as adverse effects.

It is now well documented that those patients with a deletion of a protein pathway (p53) on the 17th chromosome have disease that does not respond well to F, or FC.

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; Fludarabine, Cyclophosphamide; Prednisolone (steroids); Campath (alemtuzumab); CHOP; Peripheral Blood Stem Cell 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.)

As stated above our view is that the technology will improve the condition overall by providing longer periods between relapses..

(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).

The main disadvantages as stated above are the possible initial infusion reaction and infections during treatment.
The technology would need to be administered in a clinical setting via infusion. Current therapy is tablet based taken in the home which may be preferable for some patients reducing visits to hospital and having some control of the time the tablets are taken. However Campath, another monoclonal antibody (currently given when FC is not working, or if p53 deleted), requires 3 x hospital visits per week for up to 12 weeks.

There is some evidence of the development of other blood cancers some years after the use of the technology

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.

A*s this technology is rarely used for treatment of CLL inside the NHS (unless in a clinical trial setting) it is difficult to comment. We are aware at CLLSA that some patients received the technology privately for second or third line use. These anecdotal reports are that the technology worked well. I received the technology first line as part of treatment for NHL (subsequently diagnosis changed to CLL) and responded extremely well.*

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

None that we are aware.

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.

A Quality of Life study in 2007, published in the British Journal of Haematology (Volume 139, Issue 2, Pages 255-264) 'Quality of life in chronic lymphocytic leukemia: an international web-based survey of 1482 patients' showed that emotional well-being scores of CLL patients were lower than that of both the general population and patients with other types of cancer. Factors associated with lower overall QOL included older age, greater fatigue, severity of co-morbid health conditions, and current treatment. Among untreated patients emotional QOL scores did not change over time, which suggests that people do not 'get used' to their diagnosis.

I have included this study to demonstrate that despite CLL being an indolent cancer it is not a 'good' cancer to have. Although the technology was not a specific feature of the study, an interesting point was that for both previously treated and currently treated patients showed higher social/ family QOL scores.

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?

It would create an additional option for clinicians in the treatment of CLL. As stated above, extending the period of remission for CLL patients and their families is crucial.

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

CLL is an incurable cancer which despite its indolent nature is very debilitating to live with. Knowing there is a technology available in the western world that has been shown in clinical trials to improve the condition of CLL patients, but is unavailable in this country is very depressing.

Shorter remissions lead to more frequent treatments (and associated infections and side effects) which impact on health and well being. It is also costly in terms of numbers of drugs taken. Going from one round of treatment to another, does not lead to a good quality of life.

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

People who have to travel a long distance to hospital or have transport difficulties. There may be patients with adverse co-morbidities that may not be able to tolerate the treatment.

Other Issues

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