

Patient/carer group or patient expert statement

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 **Dr H A Pearce BSc, CChem, FRSC**

Name of your organisation (if applicable)

Chronic Lymphocytic Leukaemia Support Association (Reg. Charity No 1113588)

Are you: (tick all that apply)

- **Tick 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 organisations that represents patients with the condition for which NICE is considering the technology, if so your position in the organisation where appropriate, (e.g. policy officer, trustee, member etc)
- **Tick other (please specify)**
- **A patient who has received the treatment being considered**
- **Founder and Chairman of the sole UK patient support association dedicated to helping those with chronic lymphocytic leukaemia**

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.

Fludarabine treatment can significantly and rapidly reduce high lymphocyte counts and produce a high percentage of Complete Remissions(CR) and Partial Remissions(PR). It can reduce the tumour burden and lead to shrinking of nodes and also of an enlarged spleen.

Some patients can achieve good remissions with only minimum treatment related side effects (Quote 'Chemotherapy treatment with fludarabine was a walk in the park')

There are some reports that patients can achieve longer remissions with Fludarabine containing treatments.

(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.

Patients expect to obtain a better response than with other treatments leading to more CR/PR and possibly a longer disease free survival than with alternative treatments. Physical symptoms such as swollen spleen and nodes are reduced together with discomforts such as night sweats.

With many patients the response is quick with both short and long term benefits. Fludarabine is available in oral form as well as i.v. offering obvious benefits to patients(and their carers) with a less invasive form of chemotherapy (ie treatment at home).

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
- 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 or their family (for example cost of travel needed to access the technology, or the cost of paying a carer).

Fludarabine is heavily immunosuppressive and patients who have had this drug are more susceptible to infections. This immunosuppression affects the T cells and this effect may be irreversible meaning that the patient must then live their life with not only a cancer of the immune system but also an immune system now impaired by the treatment. This means that this treatment must be treated with the greatest of respect and ONLY used on patients who require heavy duty treatment for their form of CLL. Patients who have this drug need good support from their GP's, Consultants, Nurses etc to act quickly in tackling infections and also educating the patient in lifestyle issues to try and avoid opportunistic infections. The back up support needs to be very good.

The drug is so easy to administer (orally) and well tolerated by some that we consider that it is easy (and dangerous) to underestimate the risks involved. Fludarabine is certainly NOT the 'Walk in the park' it may appear to be. There are clear patient education issues here that need to be fully addressed when prescribing this drug.

It is noted that Fludarabine can bring on Auto Immune Haemolytic Anaemia and we have occurrences of this with some of our members. It is also noted that some patients are refractory to the drug and some patients will not get a good response when the drug is used for second line treatment. This makes the decision on timing to start treatment with Fludarabine quite critical.

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

Some patients are concerned that Fludarabine could be prescribed before it is necessary and by damaging their immune system could do more harm than good in the long term. However most patients recognise that this is a very useful drug that is effective on its own or in combination with other drugs such as cyclophosphamide or rituximab

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?

More:- Patients who are refractory to other treatments.

Patients presenting who are previously untreated and who have an aggressive form of the disease and are looking for a long disease free remission. This may particularly apply to younger patients.

Less:- Patients with a less aggressive form of the disease (ie the side effects outweigh the treatment benefits with this group)

Patients where Fludarabine is contra-indicated eg those with p53 deletion based on prognostic testing.

Comparing the technology with alternative available treatments or technologies

NICE is interested in your views on how the technology compares with current standard practice used in the UK.

(i) Please list any current standard practice (alternatives if any) used in the UK.

Chlorambucil, Prednisolone, Rituximab, CHOP

(ii) If you think that the new technology has any **advantages** for patients over other current standard practice, please describe. 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 (e.g. at home rather than in hospital)
- side effects (please describe nature and number of problems, frequency, duration, severity etc)

Better responses and longer disease free survivals.

Oral Fludarabine offers some obvious advantages in terms of administering treatment. Reduced time in hospital, more convenient for patient and carer alike, less invasive etc. (however there are some drawbacks see below)

(iii) If you think that the new technology has any **disadvantages** for patients compared to current standard practice please describe. 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 hospital rather than at home)
- side effects (for example nature or number of problems, how often, for how long, how severe).

Oral Fludarabine is very serious medication. The danger is that it is viewed as just 'another tablet' by the patient and not administered correctly at home. It requires a considerable investment in back up and support for the patient to get it right.

Of course this concern does not apply to iv Fludarabine. However, as a patient group we fully recognise the advantages of the oral route but must stress the need for good support mechanisms.

Our members report some episodes of sickness, loss of appetite and diarrhoea with Fludarabine. I can personally report a very severe reaction to oral Fludarabine which I understand was unusual (violent diarrhoea, complete loss of appetite/aversion to food, stomach pains etc etc) My medical back up and support at that time was very poor and I just accepted that what was happening to me was a 'normal' chemotherapy reaction. Fortunately the problem disappeared when I switched to i.v.

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.

No comment

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

No comment

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 comment

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?

Longer remissions.

Improved Quality of Life.

We strongly believe that this technology should be available to NHS patients

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

This would be a bad conclusion. Many of our members who have had Fludarabine have achieved good results

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

Patients who are refractory to Fludarabine

Other Issues

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

1 Fludarabine is used extensively elsewhere in the world and is most often used in combination with other agents eg Fludara/Cyclophosphamide or Fludara/Cyclophosphamide/Rituximab. Indeed it is rare to use it as a single agent in other countries. We believe that the NHS should also consider the use of Fludarabine combined with other drugs.

2 We would propose that the introduction of Fludarabine be combined with clear guidelines to Doctors on the selective use of this powerful drug. These guidelines need to take into account the latest research on Prognostic Factors to categorise and predict the future outlook of a patient's particular form of CLL and to tailor the treatment accordingly. We see this as being essential. Finally we have no doubts that Fludarabine should certainly be on the list of drugs for treating CLL which are available to NHS patients.