# NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

## Patient/carer organisation submission (STA)

## Idelalisib for previously treated chronic lymphocytic leukaemia [ID764]

Thank 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:** [redacted]

**Name of your organisation:** Lymphoma Association

**Your position in the organisation:** [redacted]

**Brief description of the organisation:** The Lymphoma Association is a national charity which provides high quality patient information, advice and support to people affected by lymphoma (lymphatic cancer). We also work with and support the healthcare practitioners who work with lymphoma patients.

(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?**

Experience of the condition varies between patients; however, CLL is an incurable condition so all patients know it is only a matter of time until they relapse and need further treament. Quality of life during periods of remission varies significantly, with some patients continuing to have symptoms despite their initial treatment or experiencing treatment-related side effects. Patients live with the knowledge that relapse and further treatment are likely to adversely affect their quality of life.

Patients with 17p deletion, especially those who are not fit enough to undergo chemoimmunotherapy, know that their life expectancy is likely to be short and that any treatments they do have are unlikely to be very effective.

## 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.**

Patients want to be in a good remission for as long as possible, so quality and duration of remission following treatment, whether this is the initial treatment or treatment at relapse, are important. They also want any symptoms and signs of their CLL, such as lymphadenopathy, to resolve. However, as this in an incurable condition, quality of life is also very important so patients want a treatment that is as well tolerated as possible, with the least detrimental effect on their quality of life.

**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?**

FCR, if it was not used as the initial treatment, is an option for a relatively small number of patients and carries a high risk of side effects, including an ongoing risk of infection. Other treatments such as chlorambucil, steroids and ofatumumab are usually easier to tolerate, but in many patients are of limited benefit, producing only short periods of remission, so that repeated courses of treatment are likely to be required, until eventually all suitable options are exhausted.

Alemtuzumab may be an option for those with 17p deletion but often causes significant side effects, including an ongoing risk of infection, so is suitable for a small number of patients and often difficult to tolerate.

## *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 treatment appears to be significantly more effective than rituximab alone, and is easy to tolerate compared to many of the alternative immunochemotherapy regimens. In particular it requires relatively little time to be spent in hospital as idelalisib is an oral agent, so patients benefit from spending time at home with their families.

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

- More effective than some of the gentler immunochemotherapy regimens.
- Better tolerated than the more intensive regimens.
- Infrequent visits required to the day unit for rituximab and, with idelalsib being oral, patients will be able to spend the majority of their time out of hospital.
- Few significant side effects so quality of life, even while on treatment, should be reasonable.

**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.**

None known to us.

## *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.**

Toxicity of many treatment regimens, particularly the risk of infection, means patients often spend significant periods of time unwell or in hospital. More gentle treatments have fewer side effects but are less likely to work well, resulting in ongoing symptoms for some patients and a short interval before further treatment is required.

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

Limited number of trials to date and even less use outside of trials. The available data relate to short-term use at present. There is little information yet about the potential development of resistance to the treatment.

**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.**

None known to us.

## *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.**

Patients who have comorbidities, often those who are older, and cannot tolerate many other chemotherapy regimens are able to tolerate this treatment, which appears to be more effective than alternative therapies that might be available to them.

Patients with poor prognositc markers such as 17p deletion appear to do as well as patients with standard prognosis CLL when receiving this treatment.

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

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

**Is your organisation familiar with the published research literature for the treatment?**

X 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.**

Limited experience to date as only recently made available via CDF.

**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?**

Yes, the important outcomes have been captured. There is however relatively limited and short-term data only because the trial was stopped early.

**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?**

n/a

**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 X 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.**

**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.**

## *9. Other issues*

**Do you consider the treatment to be innovative?**

X Yes ☐ No

**If yes, please explain what makes it significantly different from other treatments for the condition.**

The treatment has a new mode of action, is an oral and well-tolerated therapy, and appears to be particularly effective in those with 17p deletion, which is usually considered to have a very poor prognosis with standard immunochemotherapy regimens.

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

## *10. Key messages*

**In no more than 5 bullet points, please summarise the key messages of**

**your submission.**

- Generally well tolerated and suitable for patients who cannot tolerate many other therapies.
- Appears particularly effective in those with 17p deletion, who often have little benefit from other available therapies.
- Benefits versus placebo were so significant that the trial was stopped early.
- Idelalisib being oral means that patients spend relatively little time in hospital.