# NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

## Patient/carer expert statement (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, including health-related quality of life)
- preferences for different treatments and how they are given
- expectations about the risks and benefits of the treatment.

We have already asked your nominating organisation to provide an organisation's view. We are asking you to give your views as an individual whether you are:

- a patient
- a carer (who may be voicing views for a patient who is unable to) or
- somebody who works or volunteers for a patient organisation.

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 response area will expand as you type. The length of your response should not normally exceed 10 pages.

## *1. About you*

**Your name: Tricia Gardom**
**Name of your nominating organisation:** CLL Support Association
**Do you know if your nominating organisation has submitted a statement?**

☐X Yes ☐ No

**Do you wish to agree with your nominating organisation's statement?**

☐X Yes ☐ No

(We would encourage you to complete this form even if you agree with your nominating organisation's statement.)

**Are you:**

- a patient with the condition?

☐X Yes ☐ No

- a carer of a patient with the condition?

☐ Yes ☐X No

- a patient organisation employee or volunteer?
-

☐x Yes ☐ No

**Do you have experience of the treatment being appraised?**

☐ Yes ☐x No

If you wrote the organisation submission and do not have anything to add, tick here ☐ (If you tick this box, the rest of this form will be deleted after submission.)

## *2. Living with the condition*

**What is your experience of living with the condition as a patient or carer?**

I was diagnosed with CLL in 2006 at the same time as breast Cancer and they were treated immediately with 4 chemotherapy drugs Epirubicin, Cyclophosphamide, Methotrexate and 5-FU together with G-CSF with each cycle. My CLL started to progress in 2010 and since then I have experienced reduced quality of life due to repeated shingles, multiple sinus and chest infections and emergency hospitalisation for viral encephalitis. My immune system is severely immunocompromised and I receive intravenous IgG therapy every 3 weeks and take prophylactic antibiotics and anti-virals daily. These immune issues lead to social isolation from family and friends, fatigue which requires constant management and reduced flexibility with work and studying. I am currently symptomatic Stage III.

## *3. Current practice in treating the condition*

**Which treatment outcomes are important to you? (That is, what would you like treatment to achieve?) Which of these are most important? If possible, please explain why.**

I would like treatment that results in a long remission, to achieve MRD negativity, to be low in short term and long term toxicity and to preserve or preferably regenerate elements of my immune system.

It is also important to avoid secondary malignancies, neutropenia, sepsis, clonal evolution, transformation and autoimmunity where these are treatment related. I have unmutated CLL with trisomy 12.

**What is your experience of currently available NHS care and of specific treatments? How acceptable are these treatments – which did you prefer and why?**

My initial chemotherapy was extremely toxic and all given intravenously. The environment in the NHS hospital was production line orientated, disorganised and not patient orientated. I also received unnecessary bone marrow biopsy and CT scan and received no after care or prophylactic medication for my

CLL, eg anti-virals. I changed hospitals after the initial treatment. Since then I have received excellent care at a patient centred haematology unit in a hospital 45 miles away and the additional travel and expenditure is very worthwhile.

I am concerned that despite being treated with chemotherapy, I am considered treatment naïve for CLL and at present this would mean treatment with further highly toxic chemo immunotherapy. As I am 69, my options will be limited to chlorambucil-based therapies which is likely to result in a short period of remission. I will examine the possibility of trials for first line therapy in the coming months. However, my preference would be for an oral therapy, which did not require regular visits to hospital and lengthy intravenous therapy.

## *4. What do you 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 you expect to gain from using the treatment being appraised.**

Improved life expectancy, an effective response, reduction of symptoms, a lengthy remission, fewer hospital visits and consequently reduced stress, lower toxicity than existing chemimmunotherapies, and improved quality of life.

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

Lower toxicity than chemoimmunotherapy. A valuable addition for less fit patients, those with TP53/p17 deletion, those with comorbidities or the elderly.

It appears to offer the option of a maintenance therapy until a cure is found.

**If you know of any differences in opinion between you and other patients or carers about the benefits of the treatment being appraised, please tell us about them.**

None known

## *5. What do you 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 you have about current NHS treatments in England.**

Already covered above. Regular intravenous therapy in a busy day care unit can be a very stressful experience for many patients particularly when they know that they are likely to feel very unwell afterwards. Long waiting times, inexperienced phlebotomists, proximity to very poorly patients and the overall unhealthy environment all add to the unpleasantness of the experience. Oral therapies that can be administered at home are preferable.

**Please list any concerns you have about the treatment being appraised.**

**If you know of any differences in opinion between you and other patients or carers about the disadvantages of the treatment being appraised, please tell us about them.**

None known

## *6. Patient population*

**Do you think some patients might benefit more from the treatment than others? If so, please describe them and explain why.**

Patients who cannot tolerate existing chemoimmunotherapy regimens.

Patients who are very elderly.

Less fit patients with comorbidities.

Patients with TP53/p17 deletions.

Patients who relapse early for whatever reason.

**Do you think some patients 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*

**Are you 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 your experience of using the treatment as part of routine NHS care reflects the experience of patients in the clinical trials.**

The drug has only been approved for the CDF list recently so it is too soon to comment.

**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, these have been captured. The trial stopped early due to positive results and the comparator was Rituximab which is not normally prescribed on its own for treatment.

**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?**

☐ Yes ☐x No

**If yes, please provide references to the relevant studies.**

## *8. Equality*

**NICE is committed to promoting equality of opportunity and eliminating discrimination. Please let us know if you think that recommendations from this appraisal could have an adverse impact on any particular groups of people, who they are and why.**

## *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.**

Apart from points mentioned above, the fact that this is a targeted oral therapy which appears to be effective for all patient groups makes it significantly different.

**Is there anything else 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.**

- A tolerable less toxic treatment for those patients who may have run out of other treatment options or are unable to tolerate toxic chemotherapy regimens
- An oral therapy which is highly favoured by the patient community
- An effective treatment for patients with TP53/p17 deletions who have few options other than an SCT.
- A treatment which offers reduced treated related complications, hospitalisation and risk of infections
- The therapy offers an opportunity for improved quality of life and buys time until a cure is found.