# 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:** Leukaemia CARE

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

**Brief description of the organisation:**

Leukaemia CARE is a national charity; founded in 1967 and first registered with the Charity Commission in 1969; which exists to provide vital care and support services to patients, their families and carers during the difficult journey through the diagnosis and treatment of all forms of blood cancer (leukaemia, lymphoma; Hodgkin lymphoma; non-Hodgkin lymphoma; multiple myeloma; myelodysplastic syndrome; myeloproliferative disorders & aplastic anaemia).

Our current membership database stands at approximately 13,500 (this includes patients carers and members of the patients immediate family members.)

Leukaemia CARE offers this care and support through its head office, based in Worcester, its Scottish office, currently based in Motherwell and a network of volunteers all around the United Kingdom. Care and support is offered over seven key areas:

- 24-hour CARE Line and live chat (currently office hours only)
- Support groups
- Patient and carer conferences
- One-to-one phone buddy support
- Cancer campaigning and patient advocacy
- Information and booklets
- Financial assistance, in some circumstances.

Since its inception over 25 years ago our CARE-Line has taken many thousands of calls from patients, their carers, family and friends. Our website provides extensive information on all aspects of the blood cancer journey, running from diagnosis to what happens when treatment stops and includes emotional effects of a blood cancer and help for those caring for a patient. Our focus is purely on care and support for everyone affected by a diagnosis of blood cancer, simply supporting a quality of life (see http://www.leukaemiacare.org.uk).

Leukaemia CARE also works with other charities and policy/decision makers to campaign for the rights of all patients affected by a cancer of the blood to have access to and receive the best possible treatment and care when they need it.

**Organisational Funding:**

90%+ of our total funding come from our own fund raising activities, either via our members and fund raisers, legacies, grants, on-line shop, Christmas card sales, recycling exercises etc.

Leukaemia CARE receives funds from a wide range of Pharmaceutical companies, but in total those funds do not exceed more than 10% of our total income. The funds received from the Pharmaceutical Industry are received and dispersed strictly within the Guidelines as laid down by the ABPI Code of Practice, Clause 24 - Relationships with Patient Organisations.[1]

We also operate strictly within the Guidelines defined by the "Leukaemia CARE Code of Practice."[2] This Code of Conduct governing corporate funding is a commitment undertaken by Leukaemia CARE regarding our financial relationships with commercial entities and the pharmaceutical industry particularly. Both of these documents can be examined via the hyperlinks listed below, or they are available in hard copy upon request.

We pride ourselves on our independence from any external influence/undue pressure arising from any of the other stakeholder bodies operating within the same sphere of activity as ourselves – the Industry, the NHS, the DoH, NICE, the Medical Profession etc., all bodies that we work closely with but are independent from. We will maintain our independence to the best of our ability and eschew any support that could adversely impact our reputation. This fact is made clear to any drug company (or other body) seeking our advice/assistance at the time of first contact. Our Code of Conduct is also shared with them at that time.

1 - http://www.pmcpa.org.uk/thecode/InteractiveCode2014/Pages/clause24.aspx
2 - http://www.leukaemiacare.org.uk/code-of-practice

(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?**

Chronic lymphocytic leukaemia (CLL) is a malignant disorder of white blood cells. CLL is the most common form of leukaemia and is most prevalent in patients over the age of 60. The majority of patients with CLL have a slowly progressing form of the disease with long term survival, often surviving for many years (potentially over 20 years) without needing treatment. Patients often have no symptoms in the early stages and may often be diagnosed by chance when a blood test is taken for an unrelated condition.

Because CLL can behave very differently in different people, cytogenetic testing may be used to help plan treatment. The disease course can be very variable, with some patients undergoing very long remissions with treatment and others running an aggressive course with short life expectancy. Some patients will not require treatment straight away and will instead be subject to a ‘watch and wait’ protocol, whilst others may require immediate treatment.

Following a diagnosis the patient (and their carers and families) may experience the following feelings of distress: shock and disbelief, denial, anger, fear and uncertainty, resentment, blame and guilt, isolation and depression. Many of these may have a profound impact on their physical and psychological wellbeing.

Common symptoms of patients with CLL can include severe fatigue, frequent infections, enlarged lymph nodes (neck, armpit and groin), breathlessness, tiredness, headaches, unexplained bleeding and bruising, severe night sweats and unexplained weight loss. Patients may experience some or all of these symptoms.

The average incidence of CLL in England is approximately 2700. Of these patients, around 250-300 will have either of the abnormal cytogenetic changes referred to as del (17p) or TP53, which have been shown not to respond to current conventional treatment. Additionally, there may be a further group of patients for whom chemo-based therapy will be unsuitable. These patients will usually be unable to tolerate aggressive chemotherapy, e.g. fludarabine/cyclophosphamide. As such these patients may benefit from the availability of idelalisib.

## *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.**

The most important considerations from the patient perspective will include survival (preferably long-term) and a better quality of life. Another consideration for patients, their carers, friends and family is the knowledge that there may be access to effective further treatment options, should their current treatments fail.

It must be acknowledged that patient populations are not homogeneous and making generalised statements of their preferences will always carry a certain degree of risk. But we think it must be generally accepted that quality of life

and survival, normally take precedence over other outcomes for all cancer patients (including CLL patients).

If we had to hazard a guess at the most important, it would quality of life. There have been many subjective 'trade off' studies that support this notion.

**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?**

The treatments for CLL are varied and they all carry differing degrees of acceptability, including the no treatment 'watch and wait' option. Interestingly, one would think that patients on 'watch and wait' would be more comfortable and happier than patients on chemotherapy, but this is generally not the case.

Active treatment is generally preferred, and treatment with the lowest side effect profile and easier admin (oral treatment) are also preferable to the alternatives.

## *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.**

Due to the median age of patients at diagnosis many of them will be unable to tolerate the more aggressive chemotherapy treatment regimens, including FCR. As such, patients would consider the more tolerable side effect profile

of idelalisib to be a significant benefit. A further benefit to patient is that idelalisib is an oral treatment, which offers patients the opportunity to self-care and maintain their independence.

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

| Existing Treatment | Advantages of idelalisib | Disadvantages of idelalisib |
|---|---|---|
| Chemotherapy based treatment regimens | Due to the median age of patients at diagnosis (72 years) many of them will be unable to tolerate the more aggressive chemotherapy treatment regimens, including FCR. | |
| Stem Cell/ bone marrow transplant | 1. Not all patients are suitable for SC/BMT – they are often only considered for fit patients with advanced disease who have a matched donor.<br>2. The transplant process itself comes with an increased risk of multiple morbidities & mortality that would not be present with the use of idelalisib.<br>3. Idelalisib would also take away the need for such invasive treatment – which would reduce the trauma suffered by patients. | Whilst idelalisib does offer a prolonged life expectancy for patients, it is not a curative option. |
| Best Supportive Care | Best supportive care does not offer any improved survival benefit. Additionally the numerous hospital admissions associated with BSC (due to CLL complications) can have a hugely detrimental impact on patients' quality of life. | |
| Alemtuzumab | Idelalisib appears to show an improved survival benefit for relapsed patients. The treatment schedule of alemtuzumab and its associated toxicities can have a very significant impact on patients' quality of life, which the use of idelalisib would avoid. | |
| Bendamustine | Idelalisib appears to show an improved survival benefit for relapsed patients. | |

**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.**

N/A

## 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.**

- Intolerable side effects
- Regular trips to hospital – and the negative impact this has on patients' quality of life

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

N/A

**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.**

N/A

## *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.**

As outlined above, the patients who would benefit most from idelalisib are those with the del (17p) or TP53 mutations or patients who unable to tolerate the more aggressive treatment options.

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

N/A

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

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

Yes

**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.**

The treatment is not currently routinely available and as such comparison is not possible.

**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?**

We are not aware of any limitations.

However, clinical trials must try harder to evaluate quality of life end points for patients.

**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 ☐ No

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

N/A

## *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.**

N/A

**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.**

N/A

## 9. *Other issues*

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

☐ Yes ☐ No

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

Idelalisib offers a realistic, tolerable treatment option to patients who currently have very limited treatment options.

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

1. With regards to the comparator treatments: ofatumumab was de-listed from the Cancer Drugs Fund in the recent reassessments and as such is no longer an appropriate comparator.

2. There is an additional group of patients who are unable to tolerate the more aggressive treatment options. These patients must also be taken into consideration.

## 10. *Key messages*

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

1. There is presently no agreed standard of care for this group of patients, as there are limited treatment options available. As such these patients represent an area of unmet need.

2. The subgroup of patients with the 17p del / TP53 have a poor prognosis and represent a particularly high area of unmet need.

3. There is a further group of patients, who are unable to tolerate the more aggressive current treatment options, who would benefit from idelalisib.

4. There is clearly a need for a tolerable treatment which offers an improved survival benefit to both of these groups of patients.

5. Idelalisib appears to be a tolerable, oral treatment for patient which offers a statistically and clinically significant improvement in both progression-free and overall survival prospects for patients.