Appendix D – Patient/carer expert statement template

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single technology appraisal (STA)

Belimumab for the treatment of active autoantibody-positive systemic lupus erythematosus

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: Jane Dunnage

Name of your organisation: LUPUS UK

Are you (tick all that apply):

- **Yes** 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 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)
- 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.
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i) some suppression of the over-active immune system causing lupus
ii) reduction in the length of time of lupus flare
iii) increase in the length of time between flares
iv) reduction in the amount of other medications taken, esp steroids and immunosuppressive medications:

(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.

i) this is currently an incurable condition, but suppression of the over-active immune system should lead to fewer and/or less severe symptoms and the ability to lead a less restricted life.

ii) Lupus patients are normally on a number of life-long medications, some of which are toxic or have serious side effects and consequences. It should decrease the amount and number of medications necessary, but may not cut them out altogether. In particular, methotrexate and thalidomide can affect fertility; steroids have serious side effects such as osteoporosis, cataracts, weight gain; immunosuppressive medications lead to greater susceptibility to generalised infections. Serious damage to organs is caused by not only the aggressive nature of the illness itself, but by the burden of the medications, especially if treatment is necessary for a long duration, as is often the case in children and young people and those diagnosed in their 20s (LUPUS UK survey found that over 1/3rd of patients were diagnosed before the age of 35)

iii) If this treatment is licensed by NICE it could reduce the number of medications necessary, this would have a financial benefit to patients as there would be less prescription costs.

iv) Women with lupus are at highly increased risk of CHD. Although the reason for this is not currently clear, earlier and more effective treatment of lupus is likely to reduce the number of deaths and serious complications (see Haque article).

v) Pain and fatigue are the most widely experienced symptoms (92% and 86% of patients respectively reported in LUPUS UK Members’ survey): treatments which may reduce these and other symptoms are to be welcomed.
vi) Many lupus patients have found it difficult to keep their jobs because of the fluctuating nature of the illness and fatigue: Belimumab has shown both a reduction in the length of lupus flares and an increase in time between flares, and this would enable many patients to remain in employment without taking large amounts of sick leave, which inevitably leads to dismissal, or reduction in working hours and subsequent financial difficulties. (LUPUS UK members’ survey (see attached summary) showed 16% were able to work full time, 19% part time: of the 46% who were retired, 50% had had to retire on health grounds. 42% are receiving some type of benefit.)

vii) Less quantifiable is the restriction on parenting and family life which the illness can cause: it can be extremely difficult to give full attention and care to family when one is hampered by pain, fatigue and the unpredictable nature of the illness. Many lupus patients look to other family members for help with childcare but this does not alleviate the sadness and frustration that the patient feels being unable to fully play the role of a parent. (see Hale article)

viii) Nearly a third of lupus patients have mobility problems: 29% receive DLA for mobility, 84% of which are at the high level (LUPUS UK Members’ survey). Whilst it is not clear whether Belimumab will have a direct effect on mobility, if it keeps patients more active for longer periods of time this has got to be of great value not just to the patient, but also to the Benefits system.

ix) Depression can be a problem for many lupus patients (it was listed as the 3rd most difficult symptom to live with in LUPUS UK Members’ survey), sometimes as part of the illness itself, but for many the isolation, loss of work, breakdown of relationships, changed visual appearance, lack of belief in them as having an illness or understanding their symptoms by many including the medical profession, family members and employers, will cause depression. Patients also often experience the depression of a long-term illness itself. If Belimumab works to improve some of these situations it could lead to improvement in the mental health of patients as they are less likely to have to give up aspirations for careers, family life etc.

x) A reduction in time attending medical appointments would be a likely improvement from this treatment: lupus patients need to be monitored regularly and keep many hospital and doctors' appointments. When they are experiencing a lupus flare, visits to A&E departments are often necessary, where staff do not have detailed knowledge of lupus. Waiting times are very high, exposing lupus patients to the risk of infection and stress in a difficult and uncomfortable environment when they are already experiencing pain and fatigue and other serious, chronic symptoms.

xi) Another regular frustration for lupus patients is that they are unable to predict how the illness will affect them on a daily basis: this leads to problems within the family and with friends, but also within school, college and employment as the patient is not able to play a full part in these ‘normal’ activities and resentment or ostracism can result.
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xii) Compliance with medications (because of side effects, severity of medication, amount and number or just forgetfulness) can be a problem. I understand this treatment will be given intravenously at hospital and that would make it much easier for patients.

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

i) The long term nature of the technology on the patient is unclear

ii) It could trigger a worse reaction within the immune system

iii) It may cause some side effects (patients may be willing to put up with these if they are temporary or not too painful/serious)

iv) Some patients may find it difficult to attend hospital for the length of time necessary to have the infusion, but if this results in less hospital visits (esp in emergency) then they are likely to accept that.

v) This treatment works on the B cells: if the person’s lupus is not caused by this pathway, it will be unlikely that they will see an improvement, and that could cause concerns that they are not receiving the ‘right’ treatment

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

Many lupus patients are thrilled to hear of a new treatment especially for lupus and have great hopes that it will improve treatment and the outcome of the illness, if not for them, at least for other patients.

There may be concern or even suspicion about new technologies in a small number of patients, but many lupus patients have a good relationship with their consultant and look to them for their advice on treatment.

There is some concern about the funding for this new treatment and whether it will be readily available in all areas of the country: presumably NICE will give guidance on this.
### 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?

Yes to both, this depends on the nature and progression of the illness itself and the organ/systems and characteristics experienced. This drug has not yet been trialled on some lupus manifestations such as CNS, kidney and skin. Other patients have a more ‘chronic’ nature to their illness and experience fewer flares, so this drug may not help them. Fatigue and pain are the most commonly experienced symptoms, and there is not yet strong evidence about how Belimumab will affect these symptoms.

### Comparing the technology with alternative available treatments or technologies

NICE is interested in your views on how the technology compares with with existing treatments for this condition in the UK.

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

- NSAID
- Hydroxychloroquine
- Steroids
- Various immunosuppressive medications

(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.)

The technology shows promise in reducing the length of time of flares and increasing the time in between flares.

If it reduces the need for steroids and immunosuppressive medications then this will be a big benefit esp with the side effects experienced from these serious medications and sometimes the need to take further medication to alleviate side effects from the more serious ones. (see earlier comments for more detail)

Some patients will find it easier to have IV injections rather than taking many medications on a daily basis, partly because of difficulties in remembering to take medications and the cost of prescriptions for the many medications necessary on a daily basis.
Reduction in time attending medical appointments would also be an advantage.

(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).

It is difficult to predict how individual patients will react to the technology: there have been some side effects in the trials and there may be reactions to either the technology itself or the site of injections: patients will have to discuss with their consultant whether the side effects are more serious than the illness itself, or whether they are tolerable compared to the progress of the illness.

If the technology is administered within a hospital setting this will not make it difficult for the patient to use, if it eventually needs to be injected by the patient then this may be a problem for some. Hospital visits may be difficult for some if there is a long journey to be made, but if it reduces the number of hospital visits because it is more effective than existing treatments this will also be better news for patients.

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.

As far as I am aware it is not yet being used within the NHS. I know of several lupus patients who have received treatment with Rituximab, which I believe may have some similarities, and they have found that it has stabilised their condition and have been able to resume employment.

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

Not aware of its use in NHS currently.

Training needs to be given to those medical professionals who will monitor lupus patients during this treatment: the number of systems affected, fluctuating nature of the illness and the symptoms needs to be understood in order to monitor patient’s response to the treatment. There are a number of indices which monitor lupus...
damage and progress of the condition, but there will need to be training in use and interpretation.

NICE also needs to give clear guidance on its use especially on funding / commissioning decisions.

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.

LUPUS UK surveyed its members on their experience of the condition and a summary of the results is attached and have been referred to above. I have also attached and article by Liz Hale which raises important issues on quality of life, especially family life.

**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?

I believe I have covered these earlier in the statement, particularly those listed under advantages. Whilst discussing her difficulties in living with lupus, one patient (among many) has said that the hospital has become her social life as she is there so often, and has no time or energy for social activities.

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

Many people would continue having their lives seriously curtailed by this condition, because of the severity of the illness itself and the serious side effects of current medications (many patients are on a cocktail of drugs to try to reduce the impact of the steroids which were the main treatment until around 10 years ago). This illness has cost many older lupus patients their jobs, their relationships and their other hard-earned plans for the future. Now that more younger people are being diagnosed with lupus it would be a great tragedy if they also had to cope with the damage due to the aggressive nature of the condition, alongside the current heavy burden of treatments which are known to cause serious side effects and restrictions.

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

As this is not yet available on the NHS, we are not able to answer this question. I am aware from the trials that some patients experienced skin irritation at the site of the infusion, but this would be a temporary difficulty, which most would tolerate if the treatment was beneficial in the long term.
Other Issues

Please include here any other issues you would like the Appraisal Committee to consider when appraising this technology.

Training needs to be given to those medical professionals who will monitor lupus patients during this treatment: this is not an easy illness to cope with, and the fluctuating nature of the illness and symptoms needs to be understood and interpreted well in order to monitor patients’ response to the treatment.

Lupus affects people of all races, but a higher number of people from some ethnic minorities are affected, and often more seriously. This can lead to a variety of cultural issues in different races such as ability to have children, visual appearance (especially where steroids lead to excessive weight gain or permanent scarring leads to pigmentation differences) and lack of attraction leading to poor marriage prospects, suspicions about the effects of taking some medications, difficulties in explaining the illness to family members and employers.