Appendix G – patient/carer organisation statement template

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal (STA)

Vedolizumab for treating moderately to severely active ulcerative colitis [ID691]

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: [redacted]

Name of your organisation: **Crohn's and Colitis UK**

Are you (tick all that apply):

- 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)** Health Service Development Adviser
- other? (please specify)

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal (STA)

Vedolizumab for treating moderately to severely active ulcerative colitis [ID691]

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.

The aim of the treatment is to induce and maintain remission for adults with moderate to severely active UC who are intolerant of, or whose disease has had an inadequate response or loss of response to other drug treatment options. For those in this position, the potential value of the treatment is significant and could restore quality of life to the individual and avert further complications and surgery.

From trial data, it appears to be well-tolerated, with few serious side effects and has shown efficacy in patients not responding to other therapies.

(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

Clinical trial data indicates that this technology has the potential to offer patients an important treatment option where all other drug treatment options have failed. In this position, it is likely that patients' symptoms are causing them considerable distress and have a significant impact on their quality of life. Prolonged periods without control of symptoms may lead to further complications, which will then prove more difficult (and expensive) to manage. Individuals may also be faced with no other alternative but surgery and all the implications, including a potential profound psychological impact this may bring for them.

From feedback, we are aware that some patients in this situation are looking forward to an additional treatment option becoming available as soon as possible.

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

Long-term outcomes and safety profiling is still being undertaken. As a new therapy, this needs to be closely monitored, for example, through the Biological Therapies Audit. However, data from current trials indicate that vedolizumab is well-tolerated and has few serious side effects. Significantly, it also demonstrates a lower infection risk.

As an infusion therapy, some patients will be familiar with this mode of administration. Financial and other impacts will therefore also be comparable. The financial impact of not having access to this technology should also be a consideration.

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

As usage has been limited to trials to date, it is still too early to review this aspect. However, the potential value of this therapy to patients otherwise facing surgery, and for whom all other options have failed, cannot be underestimated.

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?

Longer term, this technology may be of benefit to a wider group of patients than those who have failed to respond to other drug therapies, due to its promising safety profile and lower infection risk. However, as a new technology, it is important to closely monitor usage and outcomes and conduct further analyses on specific groups of patients to identify differences.

Comparing the technology with alternative available treatments or technologies

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

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

Current standard management of UC may include a combination of aminosalicylates, corticosteroids, thiopurines, calcineurin inhibitors, TNF-alpha inhibitors and surgical intervention.

(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 of 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)

Where conventional treatment has failed and biological therapies have then made a significant difference, patients frequently report extremely high satisfaction with them – e.g. "it gave me my life back". Faced with the sometimes profound and unpredictable physical and psychosocial effects of IBD, this can be the overriding concern. However, adverse reactions and long-term complications are a serious concern with many of the current drug treatments.

(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).

Conventional therapies for UC are suboptimal – anti-TNF therapy produces remission in approximately one-third of patients, with many losing response over time - and a significant proportion of patients continue to experience flares or chronic symptoms as well as the adverse effects of nonspecific anti-inflammatory agents such as corticosteroids. The development of newer therapies is therefore an important area of research, but ongoing monitoring for safety and outcomes and an increased focus on specific groups of patients over time is required to understand the potential benefits and disadvantages of this technology in relation to other treatments and its optimal usage within the overall management of IBD.

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.

N/A

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

N/A

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.

While there is a body of research on patient and carer views of the condition and existing treatments, we are not aware of any research carried out on patient or carer

views which is directly relevant to an appraisal of this technology, but would suggest that this should be conducted and included as part of ongoing monitoring.

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?

As outlined above, this would offer a potentially highly valuable additional drug treatment option to those otherwise facing surgery and, over time, may also offer benefit to additional groups of patients if made available on the NHS.

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

If this option is not made available to patients on the NHS, those who are intolerant of, or for whom other drug treatment options have failed, will have no alternative but to face surgery and the potentially significant physical and psychological impact associated with this.

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

Patients that have difficulty with infusions would find this technology problematic.

Equality
NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that this appraisal:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which [the treatment(s)] is/are/will be licensed;
- could lead to recommendations that have 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 technology;
- could lead to recommendations that have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the Committee to identify and consider such impacts.

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