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 Crohn's disease after prior therapy [ID690]

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 & Public Service Development Manager
- other? (please specify)

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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 population being considered for this technology is adults with moderately to severely active Crohn's disease in whom the disease has responded inadequately to, or is no longer responding to, either conventional therapy or a TNF-α antagonist, or who are intolerant to either of them.

For these individuals, symptoms may include urgent and frequent diarrhoea, rectal bleeding, pain, profound fatigue, malaise and anaemia. In some patients, there is an associated inflammation of the joints, skin, liver or eyes. Malnutrition and weight loss are common. Education, employment personal relationships and social and family life may all be disrupted by the unpredictable occurrence of IBD flare-ups. The frequent and urgent need for the toilet, together with loss of sleep and the invisible symptoms of pain and continual or profound fatigue, can severely affect self-esteem and social function, particularly among the young and newly-diagnosed. For understandable reasons, a proportion of people with IBD may normalise an unnecessarily limited pattern of life, as a result of inadequate control of symptoms from poor medical management or anxiety about loss of bowel control if they venture too far from familiar environments.

In cases where diarrhoea is prolonged, or bloody and severe, water and salt loss and poor absorption of nutrients may occur, leading to anaemia, dehydration and severe weight loss. The inflammation in Crohn's Disease may lead to strictures of the bowel resulting in abdominal pain caused by partial blockage. Severe cases may lead to life-threatening complications such as complete blockage or perforation of the bowel. Crohn's Disease is often associated with anal problems such as fissure, tags, abscess and fistulas.

There is an established link between IBD and an increased risk of developing cancer, primarily in the colon. The risk of colorectal cancer increases with the extent of disease, severity of inflammation, the age of onset and duration of the disease. There is also an increased risk of small bowel cancer in some people with Crohn's Disease. At least 50% of patients with Crohn's Disease with undergo surgery within 10 years of diagnosis, and over 70% may require surgery during their lifetime.

Where other medical options have failed, this technology offers an important additional avenue to potentially alleviating the above symptoms and their impacts, restoring quality of life and avoiding or reducing the likelihood of complications, increased cancer risk and surgery.

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(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 efficacy in maintenance treatment through this technology. For those who have had to manage the symptoms outlined above, and for whom other medical options have failed, the benefits could be profound in terms of all of the above factors, as indicated under 1(a).

In relation to the impact of biological drugs, where conventional treatments have failed, patients have frequently told us "it gave me my life back".

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)

The side effects profile appears similar to other biological drugs. It is important that patients are supported to understand the full potential effects of these drugs and to make treatment decisions on the basis of this understanding, together with their clinical specialists. There is a need for further data and close observation through clinical practice to build greater understanding of the long-term safety profile.

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

Patients who have been unable to control their symptoms effectively through current medical options are understandably keen to identify other options. 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.

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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?
There is a need to conduct further analyses on specific groups of patients to identify differences in terms of benefits.

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.
As this is for use in patients who have exhausted all other treatment options, it does not make sense to make this comparison in this case. The alternative is no treatment and the impact associated with this.

(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)

The advantage is in offering an additional medical treatment option to address an unmet need. Where patients are able to achieve a stable, steroid-free remission as a result, this clearly represents a significant improvement of the condition overall.

(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 IBD 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.

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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?

This would need to be monitored closely and data submitted to the IBD biological therapies audit.

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 this technology operates using a new mode of action, this offers a significant option to those for whom all lines of current treatment have failed. Making this available on the NHS for this specific population has the potential to make a fundamental difference to their lives, if they are able to achieve remission and avert further complications and surgery as a result.

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, may have no alternative but to face potential multiple surgery and the often significant physical and psychological impact associated with this or risk longer term complications and increased cancer risk. Clinical commissioning groups will then need to make specific funding decisions on a case-by-case basis, which leads to a postcode lottery in terms of patient access.

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

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

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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.