NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Patient/carer expert statement (STA)

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

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:
Name of your nominating organisation:
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 UC three years ago but like many other sufferers I have had symptoms for much longer than that. I am the chair of the IBD patient panel at UCLH, a volunteer adviser with the Disability Benefit Service at Crohn's and Colitis UK, and a frequent contributor on the Ulcerative Colitis Forum. As well as my own experience of living with the condition I therefore have extensive experience of supporting and advising other sufferers.

Although I have not been treated with vedolizumab, I have been treated with almost all other medications that are available for UC (several forms of oral and topical mesalazine, prednisolone, azathioprine, methotrexate and infliximab). I have also taken part in clinical studies when I ran out of options. Living with UC is like living with a ticking time bomb. I know that I will flare again, it is impossible to say when, how badly, or whether I will respond to treatment. During the last three years I have had three major flares of my UC, the most recent lasting over nine months until I achieved remission through treatment with infliximab. I am treated at one of the best hospitals in the country for IBD yet it has been a struggle to access the treatment that I needed and to get my disease under control. At my worst my UC caused me to lose over a stone in weight within three weeks, become severely anaemic, and I was unable to function due to severe fatigue. I have also suffered from anxiety and low mood due to changes in my body. At my worst I needed to use the toilet to pass sometimes only blood up to ten times a night and fifteen times during the day. I have also been hospitalised. When in remission I still suffer from fatigue and I require very careful monitoring due to the side effects of azathioprine. As a young and very active person the disease has been a real drain on my life and I was only able to stay in work and progress my career thanks to infliximab treatment, for which I am in receipt of exceptional case funding from my local CCG.

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.

Vedolizumab is an extremely exciting treatment option for UC for a number of reasons. It is an additional line of treatment which is proven to be effective when all other options have failed, including steroids and other biologics such as infliximab. We are desperately in need of additional lines of treatment for UC since the current treatment options are extremely limited and have not been expanded for many years. Vedolizumab is very targeted and innovative treatment which means that it offers great effectiveness with much fewer side effects. Effectively all of the treatment options currently available for UC can lead to serious side effects and infection, which is why many patients cannot tolerate these treatments. A number of patients achieve poor response to medication which is why most patients flare at least once a year. There is a desperate need for these additional treatments.

The most important outcome for vedolizumab is that it is effective in inducing, and even more importantly maintaining clinical remission, with very few if any side effects.

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

I have been treated with almost every treatment available for UC and there are not many of these. The focus of NHS treatment at present appears to be inducing remission with corticosteroids, and maintaining this with mesalazine, or with an immunosupressant such as azathioprine or methotrexate. Steroids have side effects and are not a long term option, but many patients suffer long term damage to their health through repeated courses of steroids because they have no effective maintenance treatment. Mesalazine is often ineffective in moderate to severe cases. Azathioprine caused my immune system to disappear, but was also ineffective. Methotrexate was also ineffective for me and I understand that there is actually very little evidence that it works for UC. The only treatment that has really benefitted me has been infliximab. I had to fight extremely hard to access this and I was very lucky that my consultant supported me and my local CCG agreed to grant exceptional case funding for maintenance doses. There is a real postcode lottery in relation to accessing biologics and if you decide

not to recommend vedolizumab for UC then the postcode lottery will also exist for this treatment. Infliximab has enabled me to regain my quality of life, stay in work and avoid repeated hospital admissions. Recent draft NICE guidelines propose to shut down this treatment option for UC which is extremely disappointing for all sufferers. I will be responding separately to that consultation.

There is a misconception that surgery is an easy and cheaper option for treating UC. We would urge you to take the position that we are seeking to treat UC better with medication rather than leaving patients with no option other than radical and life changing surgery. It is also extremely important in the context of this consultation that the cost of surgery is properly considered and no assumptions are made. This includes the cost of hospital admission for up to three separate complex surgeries, follow up treatment in outpatients, stoma care, continuing medication as required, costs of the frequent complications caused by surgery, cost of fertility treatment if fertility is damaged during surgery, cost to the tax payer of the patient being unable to work and claiming benefits for up to nine months as well as the cost and complication of treating pouchitis which is a common complication with j pouch surgery. In certain cases pouchitis is treated with biologics and it would have been a saving to NHS resources for this treatment to be given to the UC sufferer in the first place prior to surgery.

Vedolizumab is not just a stop gap for certain patients to allow them to prepare for surgery a few years later, it is a long term treatment option that could facilitate long lasting remission without side effects and could help certain patients avoid surgery altogether.

The psychological impact of UC symptoms as well as surgery should be considered. UC is usually diagnosed in teenagers and young adults at a time when we are studying or starting careers, beginning relationships, having children. Surgery has a dramatic impact on a patient's life at this time.

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.

All of the above will be improved with vedolizumab. the only disadvantage is that the medication is given via infusion in hospital.

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

vedolizumab is an effective innovative treatment that is proven to have less side effects than other available treatments. It is also an additional line of treatment.

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.

n/a

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.

Concerns about current treatments: lack of effectiveness, side effects, very limited options.

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

The only concern is that some patients may find infusions difficult, however most patients will accept that the potential benefits far outweigh the inconvenience of attending hospital for infusions.

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.

6. Patient population

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

All UC patients could benefit, in particular those who have had poor response or side effects with other treatments.

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

No

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.

n/a

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

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.

A particular concern with surgery, which may be the only option for many patients if this treatment is not approved, is that fertility in young women can be affected.

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.

It is targeted at the receptors in the bowel and there is no treatment currently available that is this targeted. It is also very effective with much fewer side effects.

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.

- vedolizumab is an effective treatment for inducing and maintaining remission
- it is an additional line of treatment for UC when there are currently very few treatment options available
- it has less side effects
- it is an innovative treatment
- it has the potential to significantly improve the quality of life for many patients suffering from this condition.