NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Patient/carer expert statement (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 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: [redacted]
Name of your nominating organisation: Crohn's and Colitis UK
Do you know if your nominating organisation has submitted a statement?

Yes

Do you wish to agree with your nominating organisation's statement?

Yes

(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?

☒ Yes

- a carer of a patient with the condition?

☐ No

- a patient organisation employee or volunteer?

☐ No

Do you have experience of the treatment being appraised?

☒ 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?

In 1998 I began having abdominal pains and ill health. I was not diagnosed until 2000 with Crohn's Disease (aged 21). For those who are unfamiliar with the symptoms of Crohn's Disease, they include frequent and urgent bouts of

diarrhoea and the passing of blood and mucus from the anus. Bowel movements like this can be up to 15 times a day. Abdominal pain, chronic fatigue, anal fissures, nausea and weight loss (all of which I have suffered from extensively). The effects of anxiety caused by the condition have also plagued my life. For the next 10 years after diagnosis in 2000 I took varying doses of prednisolone along with other medications to manage my symptoms with varying but unsatisfactory successes. I tried Asacol, azathioprine, methotrexate, infliximab (which I reacted badly to), and antibiotics, but my health was never good. During these formative years of my life, the stresses of going to university, finding sustainable employment, forming relationships, socializing, etc. was challenging to say the least. My confidence was affected, I suffered with anxiety, and was constantly fighting with fatigue. In 2004 I was very unwell and during a routine colonoscopy it was discovered that I had a serious stricture in my colon that required immediate surgery. I had recently graduated university and was working in London as a sculptor/model maker. I was forced to leave this job as I was not contracted appropriately and needed to take too much time off work for recovery to keep my job. I underwent a transverse colectomy where I had 9 inches of my colon removed. Although the recovery time I was told was supposedly 6 weeks, the reality was that I was unable to work for more like 9 months. The surgery was awful and I woke up during the surgery. In hindsight I feel that I may have suffered from post-traumatic stress disorder at the time. My confidence was shot and I felt like a shadow of a man. Eventually I started work again but was still suffering with crohn's symptoms, was always tired and I got by with the help of steroids and trying to pretend that I didn't have Crohn's to the outside world.

In time I gained the confidence to do a PGCE to become an art teacher. I successfully got a job at Plymouth College of art teaching FE and HE levels. The competition was immense and the pressures of the job mixed with my disease and fatigue made those years almost unbearably stressful. I did not want my employers to know the extent of my illness as I was worried that it could be used against me indirectly if job cuts were made.

In 2010 my gastroenterologist told me about Humira and explained that as all other treatments had failed that I was at risk of being affected seriously by prolonged steroid use if I continued to take them. I was nervous about starting the drug as I am needle phobic and the possible side effects looked grim (lymphoma, etc.). However my alternative was to have my colon removed and be left with a stoma, and this wouldn't guarantee that my crohn's would not affect my small intestine in future anyway, so I agreed to take it. Before long I was able to come off steroids without any serious flare-ups. I began to feel much better than I had for years. However in 2011 I had another flare up so I had to double the humira dose. This seemed to work and after a year or so I returned to the single dose. It was during this time that the stress of my teaching job and my poor health finally got the better of me and I fell ill with serious anxiety disorder requiring medication (that I am still taking). 2 years on from then (2014) the Humira combined with my reduced stress has left me feeling almost symptom free. I still suffer with fatigue at times, but the blood, mucus and multiple bouts of rushing to the toilet with diarrhoea have ceased. I live a relatively normal and happy life now, and my last colonoscopy (a few weeks ago) showed that for the first time since diagnosis I am in remission. My consultant has suggested that as this is the first time in 17 years that I feel well that continuing humira for now is a reasonable course of action, which I agree with.

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.

Finding a treatment that cures, effectively manages the symptoms of Crohn's or better yet puts it into remission is obviously of utmost importance to me. Finding a medication that has statistically low side effects would be the ideal situation. For now the associated risks of taking Humira (or other biologics) are acceptable to me, and I am delighted to finally be in remission from my serious and at times devastating condition.

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

and why?

Generally I have been happy with current NHS treatments. I cannot say the same about treatment in the past unfortunately though. Currently I receive treatment at Derriford Hospital in Plymouth. Unfortunately there is no longer consistency in which doctors I get to see each visit which is a real shame, as the doctor/patient rapport no longer exists, and each visit I have to start from the beginning about what my history of Crohn's has been. However I feel that the current standards of care and courses of treatment are very good. I feel that I am listened to as a patient, and the help line number they have available is brilliant. I understand that Humira is sometimes difficult to be given, and it is expensive, but I feel that my consultants did an excellent job of recommending it to me and taking the necessary procedures in making it available to me. As for Alcura (the company that supplies and delivers the humira to me at home), they are very good at contacting me and arranging delivery when needed. The health care nurse that came to my house to show my wife how to administer it was excellent. It is very nice to be able to get on with the treatment independently, and not to need to be in a hospital environment every 2 weeks to receive it.

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

Vedolizumab from my understanding is a similar type of drug to Humira, which I am on. Therefore a lot of the advantages of this new drug I feel cross over with the advantages that I feel Humira offers.

Humira was the last available treatment that was available at the time of me being prescribed it. As stated above, my life was more than difficult due to the symptoms of my Crohn's disease and its refusal to respond to other treatments. Humira enabled me to come off steroids for the first time. It enabled me to live a relatively normal life. I was teaching (albeit part time) in a college without the constant worry of having to rush to the toilet at any given moment, and was able to have a child (something that I would not have been able to do if I was on methotrexate). The pain lessened in my abdomen, my fatigue lessened and the blood and mucus in my stools eventually lessened and then stopped altogether. Now I am in remission.

If Humira had failed to be successful, my consultant told me that my only last options were return to steroids (of which the side effects are many and very likely with long term use), or consider having my large bowel removed as this is where the majority of my Crohn's has historically occurred. This would have left me with a permanent stoma and no guarantee that further surgery would be needed if the Crohn's attacked a different part of my digestive tract.

Even now being in remission, I am aware that Humira in time can lose its effectiveness. Although I am trying to enjoy my remission and am hopeful that the effectiveness of Humira will enable me to remain symptom free, I do worry that should my health take a turn for the worse that there are currently no medical alternatives waiting for me should I need them. Vedolizumab seems to be an excellent alternative treatment for myself, and others who do not respond to existing treatments.

The physical and mental problems that Crohn's has caused me over the years have had a significant impact on my professional career and social life. Despite my difficulties I was a first class graduate student who was making it in the commercial art world. This ended due to my surgery. Then when I became a teacher, I was highly regarded by students and my peers, I was graded excellent by OFSTED in my teaching practice and was on the in house 'best practise observation panel'. The stress and discomforts of having Crohn's disease during the formative stages of my career have had a profound negative impact on my professional development. Having been forced to quit teaching, I now work for minimum wage as a care assistant to a man with cerebral palsy. With my education, drive and motivation I feel that if I had been administered a medication that was effective sooner I could have made much more of my life and career.

Due to my prior and current restrictions to the work I am able to do, my wife is the main income provider in our household. This meant that she was only able to take 4 months maternity leave and is worried about having another child due to my earning potential and the additional pressures it will put on her. She took on a lot of baggage when she agreed to marry me. Although I believe she is happy, I do often wish that I could be a better provider and that the strains of living with Crohn's disease didn't affect her.

Please list the benefits that you expect to gain from using the treatment

being appraised.

I expect the benefits of Vedolizumab to be equivalent to those outlined above about my positive experiences of taking Humira. It will give me and other patients with similar experiences to my own an additional treatment option where all others have failed.

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

At the time of writing this I do not know enough about the details of how Vedolizumab is administered. If it is able to be self administered then I see this as a huge benefit over infliximab, as infusions are unpleasant, time consuming and stressful to be in a hospital environment. However if it is given by infusion, then the benefit is that it is likely to be given sporadically,

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.

None known

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

Once again, at the time of writing this I do not know enough about the specific details of the differences between Vedolizumab and Humira. However, the ideal would be to find a cure for Crohn's disease. As this is not available, a drug that has the ability to put Crohn's into remission is the next best thing.

As a self confessed needle phobic, I have to admit that getting used to being injected (or having transfusions) is something that I will never truly be at peace with. Needles are invasive and I find them scary.

As Vedolizumab is in a similar group to Humira I imagine side effects may be comparable. If this is the case, then the increased risk of developing lymphoma is of concern. However it was an easy decision to go ahead with the treatment despite its risks because statistically developing lymphoma is far less than the risks associated with long term steroid use, the risks of developing bowel cancer from long term active crohn's disease and the likelihood of having further surgery.

Talking from experience as a Crohn's sufferer, any issues such as cost of going to the hospital, or time taken going to the hospital are futile if it means a chance of having relief from the symptoms of Crohn's disease.

Please list any concerns you have about current NHS treatments in England.

I simply wish for there to be a greater number of treatment options for Crohn's sufferers available to help minimize long term suffering such as those that I have endured

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

I have none whatsoever

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.

None known

6. Patient population

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

I feel that anybody living with moderate to severe Crohn's disease should be offered every chance possible to be given treatment to reduce its effects. Patients should be trialled on medications that have least risk and introduced to more powerful or harmful medications as and when it is deemed appropriate or necessary.

Do you think some patients might benefit less from the treatment than

others? If so, please describe them and explain why.

It seems that all available treatments for Crohn's disease have varying positive effects on sufferers. As it is unclear who will respond to what, it is perhaps chance that decides who will or will not be susceptive to current and future treatments.

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

Are you familiar with the published research literature for the treatment?

☐ 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.

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?

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?

Are you aware of any relevant research on patient or carer views of the condition or existing treatments?

☐ Yes ☐ 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.

I feel that as there are only a limited number of treatment options available to Crohn's sufferers, and so many patients respond poorly or not at all to existing treatments, denying the approval of Vedolizumab would be a form of discrimination to the group of patients who are running out of, or have run out of, further treatment options.

9. Other issues

Do you consider the treatment to be innovative?

☐ No

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

Is there anything else that you would like the Appraisal Committee to consider?

It is hard to put into words how much of a benefit to my life Humira has been to me. I have spent my entire 20s and early 30s feeling like half a man, living my life around where the nearest toilet is, paranoid about not finding one in time, not wanting to be in social situations, suffering terribly with exhaustion whilst trying to develop a career, pain and discomfort, the horror of filling the toilet with blood multiple times a day, being on medication that I know is damaging my body, undergoing terrible surgery and the slow painful recovery, explaining to girlfriends about my condition and hoping that they won't be put off, the knowing that each flare up I have was leaving scar tissue in my bowel and increasing my risk of bowel cancer, and much more.

If Humira becomes ineffective to me in future, my life will likely return to the misery that it was. If Vedolizumab is given the go ahead it could potentially save others who have been on similar paths (or myself should Humira fail to be effective) from living the challenging and often miserable lifestyles that I have experienced.

10. Key messages

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

- Crohn's disease is a debilitating, painful, embarrassing and miserable condition to live with. Patients should be offered as many treatment options as possible to treat it, as current medications are often ineffective.
- Crohn's disease does not just affect the mechanics of the bowels, but it can have negative impacts on mental health, careers, relationships, social lives and overall quality of life.
- Vedolizumab offers an additional treatment option to those who have failed to respond to previous medications and are running out of ways to manage their symptoms and live a 'normal' life.
- The risks of possible side effects of Vedolizumab are greatly outweighed by the risks of long-term steroid use, surgery and prolonged flare-ups that can lead to bowel cancer.
- Having experienced the life changing benefits that Humira has given me (achieving full remission from Crohn's disease), I know how incredible Vedolizumab could be to Crohn's sufferers who have not responded to currently available treatments. I feel like I have a life finally.