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: Kameron Singh
Name of your nominating organisation: Crohn's and Colitis UK
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?
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☐ Yes ☐ X No

Do you have experience of the treatment being appraised?

☐ X Yes ☐ 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 have pan ulcerative colitis which, since my diagnosis in June 2000, has been extremely difficult to control. I was steroid dependent and all conventional UC therapies failed – including anti TNF (Infliximab). Long term steroid use resulted in osteoporosis at age 28. I was house bound for many years due to UC and was unable to work. Quality of life was zero.

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.

Achieving and remaining in remission from UC is mine and every other UC sufferer's goal. This would have to be without the use of steroids. Remission is important as otherwise I would not be able to work. I would not be able to leave the house. Basically, I would not have a life. Avoiding life-altering surgery is also extremely important. I would want to avoid this as I have seen the many complications this can bring about as well as the psychological trauma that is often apparent.

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

Current NHS treatments did not work for me, nor for many of my fellow UC sufferers, including close friends. Steroids still tend to be given as a first line treatment which is unacceptable. I have developed serious life-long side effects due to this practice, which inevitably result in an additional burden on the NHS. Retention enemas are expensive and invariably deliver poor results, as was the case with me. I had a similar experience with oral 5 ASA (aminosalicylates) treatments – mesalazine and balsalazide. These treatments are old and do not bring about improvement in many patients. The same applies to Immunosuppressive treatments. I have been treated with the thiopurines azathioprine and tacrolimus, with poor results. This treatment, by far, is better than all current NHS treatments, with the added bonus that unlike current treatments, has not resulted in any side effects. I have also previously

been treated with the anti TNF Infliximab. This treatment had no effect on my condition and treatment was subsequently discontinued.

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.

I began this treatment in January 2009 and it has given me my life back. I have been in an uninterrupted remission for the entire past 6 years. I am no longer house bound. I am able to go to work. It has taken away the crippling pain and allowed me to sleep at night. It has stopped me bleeding every time I have a bowel motion. I am no longer having to ensure I am always near a toilet due to diarrhoea, which is a defining feature of UC. I can travel and see friends and family, something I could never do before I had this treatment. My mental health was suffering from years of flaring from UC and this has been completely resolved since I went into remission on this treatment. I am no longer reliant on harmful steroids and this alone has been a benefit that is completely immeasurable. In the past I have spent weeks in hospital wards with serious flare-ups of UC. This has never happened in the six years that I have been on this treatment.

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

None

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.

Current NHS treatments are failing many UC sufferers. They are being forced to live a very poor quality of life. Taking more than 10 tablets a day in addition to uncomfortable retention enemas, is not uncommon. The serious and often permanent impact steroids have is widely documented.

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

None

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.

N/A

6. Patient population

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

Many patients are intolerant of immune suppressive drugs. They often suffer severe reactions and treatment has to be discontinued. These patients would clearly benefit from this treatment.

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

No, I do not believe this would be the case.

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.

I am currently receiving this treatment as a patient enrolled in a clinical trial.

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?

I have been on this clinical trial for almost 6 years. There has been extensive collecting of data during this time. Regular and comprehensive patient surveys and daily symptom recording has taken place. Several colonoscopies have also been carried out during my treatment, providing irrefutable evidence on the efficacy of the treatment.

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?

No side effects have been evident to me during my treatment.

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.

None

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.

This treatment is innovative in that it works differently to the 5 ASA drugs and enemas that are currently widely used. When I was flaring with this illness, taking tablets by mouth was a pointless exercise. I was not able to properly absorb the tablets and they simply passed straight through. Similarly, with enemas, the inflammation was such that I was not able to hold the enema in long enough for it to have any beneficial impact. It needs to be held in for some time for it to have any effect. This was a near impossible feat for me and many other people I have spoken to about these treatments. At best I could only retain the enema for 5 minutes before the urgency to release it became too great. This rendered the expensive treatment useless.

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

In the years before I was given this treatment. I spent practically all my life in hospitals. I was malnourished and was seriously underweight. I developed eye and ear problems as well as osteoporosis due to steroids. I developed potentially life threatening neutropenia due to immunosuppressive treatments,

currently given to UC patients as NHS treatment for UC. All of this is a thing of the past since I began on this treatment six years ago.

10. Key messages

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

- Ground breaking new treatment which works when many treatments have failed
- It will save thousands of people from a life of misery and pain
- I have experienced no side effects
- Mental well being is a huge benefit
- Could potentially save the NHS thousands from current treatment complications