NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal (STA)

Rituximab in combination with corticosteroids for treating anti-neutrophil cytoplasmic antibody-associated vasculitis

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: MISS LISA RANYELL

Name of your organisation: VASCULITIS UK

Are you (tick all that apply):

- X 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?
- X 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)
- other? (please specify)

I broadly agree with the statement already made by Vasculitis UK, but just wanted to add a personal statement about my own experience.

I am a 44 year old female from Leicestershire, working part-time as a Complementary Therapist.

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I started having nosebleeds, sores and crusting in nose in summer 2005 which were a mystery to my GP and appeared to disappear by themselves within 6 months. The following summer they came back with a vengeance along with ear discharge, pain in face, ears and jaw along with fatigue weight loss and other symptoms. After many hospital visits I was finally admitted and diagnosed with GPA (Limited Wegners Granulomatosis) in October 2006. I started on high dose prednisolone and cyclophosphamide, along with a raft of other drugs, at some points taking almost 40 tablets a day and unable to work for 4 months.

During this initial period I suffered with saddle nose, completely collapsing bridge of nose, Bell's palsy, complete deafness in right ear, and showed lesions in lungs and a mass (granulomas) close to right temporal lobe. Despite treatment over next 2 years with 13 pulses of cyclophosphamide, a year of methotrexate, and constant prednisolone never less than 20mg my symptoms progressed and I developed a large colonisation of Staph Aureus in my lungs.

During this 2 year period I spent a total of around 12 weeks as an inpatient in hospital along with numerous x-ray, CT and MRI scans and weekly outpatient appointments, I was able to work sometimes but had at least 10 months off work during this period. In general I was very poorly and only the prednisolone was keeping me going.

In 2008 i had a major flare up and was admitted to hospital and checked for Brain Tumour, TB and Meningitis at various times along with all the lovely test procedures that come with these! Just as it looked like things were getting dire, my consultant conferred with a different hospital and they recommended Rituximab. I had my first infusions in November 2008.

I was feeling much better within 2 months, and had no adverse reactions to the infusions. I had another 2 infusions 6 months later and was completely off steroids within a year.

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I have had minor flare ups since then when we tried to lengthen gap between infusions but I am currently just having one infusion every 6 months as maintenance.

I have not had any hospital admissions as an inpatient during the last 4 years since I started on Rituximab, I take very few tablets and my quality of life is hugely improved. I manage to work between 24 and 32 hours per week.

I strongly believe that this drug saved my life.

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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.

I believe the main advantages of this technology are:-
- it is less toxic and generally better tolerated than alternatives.
- less side effects in most people.
- does not affect fertility.
- reduction in hospital admissions.
- less monitoring of patients.
- reduction in number of drugs required by most people.
- improved quality of life.
- less relapse.

(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.

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What do patients and/or carers consider to be the advantages and disadvantages of the technology for the condition? (continued)

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

Disadvantages:-
-some patients react to the infusions.
- long infusion time.
- problems have been reported with white cell counts and ig levels.
- patients being denied the drug due to cost.
- long term side effects not yet identified.

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

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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?

Comparing the technology with alternative available treatments or technologies

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

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

(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 in 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.)

(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

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home)
- side effects (for example nature or number of problems, how often, for how long, how severe).

Equality and Diversity

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

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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.

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

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.

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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?

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

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

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