Deborah Shipman.

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Patient/carer expert statement (STA)

Omalizumab for treating previously treated chronic spontaneous urticaria

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: Mrs Deborah Shipman
Name of your nominating organisation: Allergy 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?
-

☐ Yes x 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 have suffered with the condition in various forms since early childhood but it became very bad about a year ago when I developed hives over all of my body from head to toe which were present all the time. There was no let up in the condition. Current medication is helping short term.

I had rashes all over my body with burning, extreme itching, swollen wheals which coerced into larger patches which would cover whole areas of my body. I was completely unable to function. At that time my husband had to take over everything, the hives were unbearable; I could not sleep and spent my whole time applying cold flannels and cream to try and calm the itch and burn. There was no way I could play with my 4 year old son, cuddle him or care for him, neither could I hug my husband never mind any further marital relations. I was as good as house bound. Clothing can exacerbate the symptoms due to pressure from waistbands, bra straps, and irritation from labels seams etc, as did heat. I was like this for weeks, crying every day, practically suicidal as I could not stand another day of this "torture". I eventually was given steroids which gave me relief for a week, then back to the symptoms, then another week of steroids to be told that was all I could have (2 weeks worth of steroids). After this I struggled on with one a day antihistamines, waiting and waiting for a hospital appointment. (this took weeks). After seeing the consultant I wasn't much better off as I was prescribed antihistamines which did not work even at 6 times the usual dose. I was then put on steroids which thankfully stopped the hives. The side effects were not good, mood swings relative to manic depression, plus weight gain. I was then put on Ciclosporine at my own request which does not relieve all the symptoms but helps a lot. At the prescribed dose of 175mg I had nausea and upset stomach, stomach pain, extremely raised blood pressure, pins and needles in arms and legs. Also facial hair growth, burning in my hands and feet, tiredness, raised Bilirubin in my liver, continuation of mood swings, and a general feeling of being unwell. I have lowered my dose and the side effects are not so bad now. At this point I am waiting to see what comes next, praying for remission

and terrified of the future with hives and no suitable medication to control them.

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.

I would like to be able to manage my condition so I did not need constant visits to doctors/hospitals, constantly changing medications, and never knowing what's next on the agenda. I would like to "get my life back to normal" and be able to carry out everyday tasks, work and participate in family life and have some sort of social life.

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

GP's in my experience have very poor knowledge of this condition. Diagnosis is difficult and a very slow "hit and miss" process. Doctors just don't know enough about the condition. Even the consultants I have seen don't know enough and have varying ideas on treatment. None of the consultants or doctor providing my treatment to date has had knowledge of the drug being appraised. There is a lack of knowledge and consistency of treatment. Long waiting times for appointments especially the initial consultation exacerbate things, as the whole time a patient is going untreated they are suffering this debilitating illness. I was offered antihistamines up to 4 x licence dose which did not help, then Montelukast etc. I was on steroids for months which helped the illness but caused side effects. I then went on to Ciclosporine which has been the best drug to date. Although this has a wealth of side effects many of which I have suffered. It is also I am told only a short term option for Urticaria. Omalizumab would seem the next step for me but I have been told it is unavailable. I don't feel that any of the drugs are really acceptable as they are not licensed for this condition and for many there is no actual research or evidence that they actually work. Drugs are often given on a "suck it and see" basis. The patient doesn't know if they will get any relief from their symptoms or what side effects they are going to experience. In a perfect world this is unacceptable. I was most concerned when one senior consultant I saw said I

could take Methotrexate even after my usual consultant had said it wasn't safe for me, the senior consultant also told me long term Ciclosporine is fine to take despite my previous consultants telling me it was only used for a few months and to wean off it. This is an example of the lack of continuity patients experience in their treatment. Personally I prefer the Ciclosporine being to me the lesser evil overall, but am mindful of the long term side effects.

4. *What do you consider to be the advantages of the treatment being appraised?*

From speaking to many patients who take the treatment being appraised it seems to give them back their quality of life enabling them to get back to normal. Many have complete remission of the illness, others only slight symptoms. I have spoken to a lot of people who because of this medication have been able to go back to work, the gym, cycling, hobbies and a normal social life. There do seem to be side effects but no worse than any of the other treatment options.

It is a once a month injection so there are no tablets to take daily which rules out problems such as nausea and stomach upset from ingestion of medication. As far as I am aware Omalizumab does not require blood monitoring either making it more convenient than immunosuppressant's.

A drug that can put this disease into complete remission would be a godsend for me and many others for whom there are limited or no other treatments that work. It would also help psychologically as it takes away the worry factor knowing that the illness is under control.

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 would hope that If the treatment worked for me and put my illness into remission I could get on with my life as normal with no more worrying about "what's next" Just to put the illness to the back of one's mind instead of it being constantly in ones thought would be a massive benefit. A once a month trip for the injection being all that is needed.

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

Advantages would be that it is a long term treatment; there are no other effective long term treatments available for those that do not respond to antihistamines. It appears to have fewer day to day side effects. No need to take daily medication/tablets. No need for regular monitoring of blood, blood pressure, urine etc.

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.

I don't know of any.

5. What do you consider to be the disadvantages of the treatment being appraised?

It could be an inconvenience to have a monthly injection involving travel but no more so than monthly trips to hospital for blood monitoring and medication etc.

Continuity of treatment would be a factor. The drug is currently prescribed for 6 months at a time and then another application has to be made which

could take up to 5 months. This is unacceptable as it puts the patient back to square one, causing pain, suffering and depression. A patient with the disease long term could find they are 6 months on the drug followed by 2 to 5 months off it, and then back on for 6 months and so on. If a person's illness continues for years this would be a horrible situation to endure.

The fact that it is an injection may be a disadvantage, I myself dread needles but would be willing to cope with my fears to control the hives as most would. All the drugs used for this illness seem to have quite severe side effects so this would be no worse. I have some concerns about recent reports from the FDA about the higher risk of mini stroke, heart attack, chest pain and clots in the lung and veins. I would like to know more about this risk.

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.

My concern is lack of continuity, consistency and knowledge. Plus no psychological back up for treatment. Very slow diagnosis and long waiting times for referrals. Patients being left on dangerous medications long term such as steroids and Ciclosporine.

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

The recent discovery of the small risk of stroke, heart problem etc.

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.

Some people are concerned with it being relatively new and untested. But I know that most people (I have spoken to) don't care about the side effects they just want relief from a horrendous illness. People are desperate to get rid of the hives no matter what it takes.

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 think the people to gin most benefit would be those who have suffered either long term and/or who suffer very bad hives on a daily basis, those that don't respond to antihistamines. Those people who only seem to get relief with steroids or autoimmune drugs these would be most suited to this treatment.

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

It would not be suitable for people who have minor hives or suffered for less than six weeks. I think if a patient's hives can be controlled with other drugs such as antihistamines it would not benefit them to use this treatment as it would not be worth the risk of side effects, having to have injections, travel etc.

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

Yes x 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.

7. *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.

no

8. *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 biological and specifically licensed for Urticaria (and Asthma etc). There are currently no medications licensed for Urticaria.

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

Patients continued access to the treatment is very important. I understand that after a certain period of time say 6 months that treatment has to be stopped to see if the urticaria has gone. But once this has been done treatment needs to be available to restart immediately otherwise the patient will suffer terribly. The fear of knowing that treatment is only there for 6 months and then will be

taken away all be it temporarily is awful. I spoke to one lady who had to wait 5 months between treatments. During this period she had to go back on steroids which are not ideal. A system needs to be in place where the medication is approved for longer periods of time on the agreement that breaks are taken to see if the condition has gone into spontaneous remission and no longer needs treatment. If remission is achieved it is easy enough to stop treatment but if a patient is suffering badly it is totally unfair to expect them to wait months for further approval of the drug.

9. *Key messages*

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

- Early diagnosis/prompt referrals
- Continuity of treatment
- Equal availability of treatment for patients
- Education of healthcare providers
- Psychological back-up care

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