NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Patient/carer expert statement (MTA)

Abatacept, adalimumab, etanercept and tocilizumab for treating juvenile idiopathic arthritis (including review of TA35) [ID738]

Thank you for agreeing to give us your views on the treatment(s) being evaluated by NICE in this appraisal and how it/they 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(s).

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. If you think your response will be significantly longer than this, please contact the NICE project team to discuss.

When answering the questions from section 3 onwards, please make sure to specify which treatment (s) you are commenting on.

1. About you

Your name: [redacted]
Name of your nominating organisation: National Rheumatoid Arthritis Society
Do you know if your nominating organisation has made a submission?

[redacted] Yes ☐ No

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

[redacted] 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?

[redacted] Yes ☐ No

- a carer of a patient with the condition?

☐ Yes [redacted] No

- a patient organisation employee or volunteer?
-

[redacted] Yes ☐ No

Do you have experience of the treatment (s) being appraised (that is, those included in the title)?

☐ Yes [redacted] No

If yes, please tell us which one(s)

N/A

If you wrote the submission from the patient organisation 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 systemic JIA aged 2 but it is likely I was born with the condition. The JIA was very aggressive during my childhood and treatment options were limited so I sustained joint damage early on. It got to the point that my parents were advised I would most likely need joint replacements in adulthood. As a child I remember hospitals and my bedroom. I wanted to join in with my friends but I would end up hurting even more and so I would retreat to my bedroom. Books became my escape and I would often pretend I was one of the characters, free of the JIA. I didn't understand what was happening to me, or why. I would get very tired at playtimes so would sit and watch. At lunchtime, I would go to the medical room to rest. I couldn't always sleep because of the pain and so I was prescribed medication to help me sleep. The problem was I would then fall asleep in school.

I didn't like my medication. Due to limited treatments, I started off on adult doses of aspirin before going onto prednisolone and gold injections. I hated the taste of prednisolone and so my Mum would give them to me with lemon curd. Even now I can't have lemon curd without tasting the bitterness of prednisolone. I hated my gold injections and would often hide when I saw my notes being picked up by the nurse. I know from talking with my Mum that it impacted her because she had to watch me being almost restrained so I could be given my injection. Bear in mind this was 30-35 years ago but it has stayed with us as a family.

Schooling was a difficult time as I had a lot of time off for hospital appointments and illness. I wore orthopaedic shoes and callipers as well as work/rest splints. This set me apart from my peers and I did experience bullying within secondary school. I felt I couldn't go for sleepovers because I was embarrassed about my splints and medication.

Teenage years are when you define yourself as an individual but living with JIA brings hurdles. By this point I was on methotrexate but the nausea was horrible. I couldn't always face food. I felt different to my peers, not being able to wear 'nice' shoes, wearing trousers because I didn't want people to see my swollen knees. Not being able to do physical activities, especially contact sports. Not being able to socialise because of the tiredness. Watching my peers get boyfriends and wondering if that could ever be a reality for me. Self-esteem was a huge issue and I experienced bouts of depression but it wasn't something that was really picked up on until I was 25.

Going onto adulthood, while my JIA is now in remission, I still live with the results of aggressive disease. I live with joint deformities, I still require surgery, and I have gastro-oesophageal reflux. My mental health is still affected and I have bouts of depression for which I take duloxetine. Despite all this, I remain positive and support those living with a long term condition. I run a support group, have recently trained as a facilitator for self-management training and I am getting married later on this year.

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.

The most important outcome is that the JIA is controlled and limits joint damage. For me, the joint damage brings consequences that will stay with you for the remainder of your life. Having joint replacements at a young age is problematic due to limited number of revisions you can have in a lifetime. There is also the associated nerve damage as well as the psychological impact.

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

My JIA is in remission and so my current treatment is Naproxen with the occasional Depo-Medrone injection. These treatments work in keeping the JIA inactive but there are times I need to increase pain relief because of

associated joint damage. I have found hydrotherapy very beneficial because of the heat and non-weight bearing but it is limited on availability. I see my rheumatologist every 12 months and I do have access to physiotherapy but I feel there is a need for a greater understanding of JIA.

4. What do you consider to be the advantages of the treatment(s) 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(s) being appraised.

All of the above are definite benefits of the treatments being appraised and this includes the family as JIA impacts them too. In regards to where the treatment has to be used, this is an important consideration, one that needs to involve the child and family. If a child needs to travel for treatment this can impact further on education plus the social and emotional needs of the child as well as the family. There may also be financial implications.

Please explain any advantages for the treatment(s) being appraised compared with other NHS treatments in England.

From personal experience, Methotrexate is considered the gold standard treatment and so is widely used. Intra-articular steroid injections can be very effective but again from personal experience are painful and evasive. If standard DMARD therapy fails then having the option of biologics is a huge benefit, especially when you compare it to long term use of steroids, and the

associated complications. Having spent 15 years on steroids, I am aware of the implications having experienced thinning of the bone, stomach ulcers, menstrual and skin problems.

If you know of any differences in opinion between you and other patients or carers about the benefits of the treatment(s) being appraised, please tell us about them.

I am not aware of any differences of opinions.

5. What do you consider to be the disadvantages of the treatment(s) 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

It may be difficult for the child to come to terms with injections, as well as their parents. It is hard to watch your child being injected, or even having to give the injection. Overall though, with explanation and support as well as the benefits of feeling better will outweigh any negatives.

As mentioned before if a child needs to travel for treatment this can impact the family as well as education. There may also be financial implications.

I had to travel 32 miles for treatment and we did not have a car. I also had 2 younger brothers and so my parents had to ensure they had care. As it was a long day, I would not be in fit state to attend school.

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

Like any person taking medication, I've had concerns about the toxicity of a drug. How would it affect me long term, what are the side effects? Being given the information can be reassuring but at the same time frightening

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

Again, it would be the side effects. It is a natural concern and one that needs raising. However, from personal experience, I know the positives of having inactive disease.

If you know of any differences in opinion between you and other patients or carers about the disadvantages of the treatment(s) being appraised, please tell us about them.

I am not aware of any differences in opinion.

6. Patient population

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

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

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

Are you familiar with the published research literature for the treatment(s)?

☐ 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(s) 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 the assessment of the treatment(s) in clinical trials?

If already available in the NHS, are there any side effects associated with the treatment(s) being appraised 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.

It could impact low income families, particularly if they have to travel for treatment. It could also impact single parent families who may not have access to child care when travelling for treatment.

9. Other issues

Do you consider the treatment(s) being appraised to be innovative?

☐ Yes ☐ No

If yes, please explain what makes it significantly different from other treatments for the condition. (If this applies to more than one treatment that is being appraised, please give reasons for each one.)

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 statement.

- Getting control of the disease earlier is vital as this minimises joint damage and further implications
- There is a need to recognise the physical and emotional impact of failing treatments. It is a frightening prospect to see the disease progressing
- It is important to remember the family unit lives with JIA and shares the impact
- Living with JIA can impact financially, more so if the family are not in receipt of benefits such as disability living allowance.