

**NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE**

**Single technology appraisal (STA)**

**Tocilizumab for the treatment of systemic juvenile idiopathic arthritis**

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: Helen Copeland**

**Name of your organisation: National Rheumatoid Arthritis Society**

**Are you (tick all that apply):**

- 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?
- 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)

National Institute for Health and Clinical Excellence

Patient/carer organisation statement template

**Single Technology Appraisal of Tocilizumab for the treatment of systemic juvenile idiopathic arthritis**

**NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE**

**Single technology appraisal (STA)**

**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 expect the technology to reduce the aggressive nature of SJIA, therefore limiting the joint damage and deformity that is caused.

(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.

JIA and sJIA can be aggressive in nature, meaning that it can affect a number of joints and in the case of sJIA, the whole body. The potential benefits of using Tocilizumab vs standard (failing) treatment is relieving pain and inflammation, reducing or preventing the deformity of joints, as well as restoring use and function of the joints to promote optimal growth, along with physical activity such as 'childs play'.

This in turn would benefit the childs quality of life. 'Childs play' is essential for helping children reach important social, emotional, and cognitive developmental milestones as well as helping them manage stress and become resilient. If a child has uncontrolled JIA or sJIA this can limit play and may lead to isolation from their peers.

On a longer term basis, the potential benefits of using Tocilizumab vs standard (failing) treatment would be to improve mental health. If a child has damaged/deformed joints this often leads to ongoing surgery, extending into their adult life. This would put limitations on their independence. Having time off from education and employment results in social exclusion as well as pigeon hole them in stigmatised groups.

National Institute for Health and Clinical Excellence

Patient/carer organisation statement template

**Single Technology Appraisal of Tocilizumab for the treatment of systemic juvenile idiopathic arthritis**

**NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE**

**Single technology appraisal (STA)**

Overall this affects mental health, potentially causing neuroses, including anxiety states, phobias and long term depression

**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).

One disadvantage would be if the patient would need to travel long distance for treatment. If a parent has had to leave work or reduce their working hours to look after the child, there may be a reduced income. Travelling long distances could impact the financial situation as well as the family unit, particularly if there are siblings.

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

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.

National Institute for Health and Clinical Excellence

Patient/carer organisation statement template

**Single Technology Appraisal of Tocilizumab for the treatment of systemic juvenile idiopathic arthritis**

**NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE**

**Single technology appraisal (STA)**

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

**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?

**NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE**

**Single technology appraisal (STA)**

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.

**NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE**

**Single technology appraisal (STA)**

**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?

Given the aggressive nature and severity of disease in sJIA, including a higher mortality rate, having access to this technology on the NHS would give reassurance to parents that there is a different mode available if a standard treatment fails. It would also mean that other medication, in particular steroids which are extremely damaging taken long term can be reduced.

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

Implications include severe joint damage, the need for ongoing surgery into adulthood, intermittent periods of leave from education and employment and isolation from society.

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

**Other Issues**

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

**NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE**

**Single technology appraisal (STA)**

National Institute for Health and Clinical Excellence

Patient/carer organisation statement template

**Single Technology Appraisal of** Tocilizumab for the treatment of systemic juvenile idiopathic arthritis