NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Patient/carer organisation submission

Abatacept, adalimumab, etanercept and tocilizumab for treating juvenile idiopathic arthritis (including review of TA35)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, and including health-related quality of life)
- the acceptability of different treatments and how they are given
- expectations about the risks and benefits of the treatment(s).

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 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 say which treatment (s) you are commenting on.

1. About you and your organisation

Your name: [redacted]

Name of your organisation: National Rheumatoid Arthritis Society

Your position in the organisation: Chief Executive

Brief description of the organisation: NRAS is the only patient led organisation with a specific focus on Rheumatoid Arthritis and since 2013 this includes JIA, through launch of our new service for children, young people and families in 2014 called JIA@NRAS. We provide a complete service for patients, families and carers and:

- Ensure that people affected by RA/JIA have the information, tools and support to be able to access high quality care and services best suited to their individual needs and preferences.
- Do everything we can to raise standards of care and ensure equitable access to high quality care and treatment for all with RA/JIA.
- Pioneer innovative and effective ways to support all those living with RA/JIA to lead full and active lives with maximum well being, able to pursue their individual goals.

We have a membership of over 5,000 and have a wide range of income streams to support our activity. We receive no statutory income. The largest income streams are from trust and grand giving bodies and from our events programme. We receive some education grants from industry but this income is capped at a max. of 15% of our total income.

(For example: who funds the organisation? How many members does the organisation have?)

We are asking for your collective view as an organisation and will be asking patient experts for their individual input separately. If you have the condition, or care for someone with the condition, you may wish to complete a patient expert questionnaire to give your individual views as well.

2. Living with the condition

What is it like to live with the condition or what do carers experience when caring for someone with the condition?

I have lived with rheumatoid arthritis for over 30 years and so whilst I do not have JIA, I do live with severe inflammatory arthritis and so have a huge experience of dealing with chronic pain, fatigue, the impact on daily life, the challenges of working with such a disease and needing to take serious medication for life, as well as the effect on my family, friends and colleagues.

Our volunteer, Helen Berger, who will be attending the Appraisal, will be submitting a personal statement and she has lived with JIA since babyhood.

3. Current practice in treating the condition

Which treatment outcomes are important to patients or carers? (That is, what would patients or carers like treatment to achieve?) Which of these are most important? If possible, please explain why.

Treatment of JIA, like RA, has been revolutionised by the advent of biologics. In JIA, the healthcare teams aim for nothing less than remission and the good news is that this is now possible in a way which was not the case before the widespread use of Methotrexate and the introduction of biologics. The most important outcome for patients and families is the complete suppression of inflammation leading to the child or young person being able to return to normal life, with a complete absence of symptoms such as pain, stiffness, fatigue, etc. and enabling that child or young person to behave as normal, attend school, take part in normal sports, leisure and social activities and grow normally. JIA can affect growth hormone and in trials in patients with Systemic JIA, Tocilizumab, for example, has demonstrated that patients can experience significant catch up growth. Many young people are successfully controlled on Methotrexate, but where this does not work, is not tolerated or is contra-indicated, the use of the biologic treatments which are the subject of this appraisal, have shown to be life-changing for many and vital to enabling the child/young person to lead a normal, independent life, free from pain and disability.

What is your organisation's experience of currently available NHS care and of specific treatments for the condition? How acceptable are these different treatments and which are preferred and why?

In 2012/13 we visited 32 units, both adult units which treat children and specialist paediatric rheumatology units to interview teams, managers, families and young people in order to map access to services for children and young people with JIA across the UK. At the end of 2013 we published our 90+ page report – A Focus on JIA – which showed that service delivery models vary quite widely and access to best care is not universal. Our report can be accessed here

There are often delays in getting a child or young person diagnosed, public awareness of the fact that children get arthritis is low and GPs rarely see the condition and so are not always familiar with the early signs and symptoms. The reality as stated in the previous question is that treatment today is much better than it used to be, and caught early, outcomes are very good for many. There will always be some for whom current best practice treatment is ineffective and this is why it is extremely important to continue to invest in research to address this unmet need.

4. What do patients or carers 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
- Ability to remain in full time education
- Ability to attend university or further education
- Ability to get a job and remain working
- Ability to attain independence from family

All of the above are benefits of the treatments being appraised. It is extremely important to allow maximum clinical freedom in use of biologics as these will always be initiated by a specialist in paediatric rheumatology who will know best at what point in the child/young person's pathway a biologic needs to be introduced to ensure best outcomes, reduction of the potential for disability and growth retardation and total suppression of inflammation.

Please list the benefits that patients or carers expect to gain from using the treatment(s) being appraised.

The impact on the whole family when a young person has JIA can be immense. It can be very disruptive to family life, cause financial hardship if a parent has to either stop work or take extended periods of time off work to care for the child. It can lead to sibling jealousy and feelings of isolation leading to depression and other mental health problems for not only the individual with JIA but others in the family. It can cause parents to split up and all of these issues can be costly not only to the family concerned, but to wider society through needing to access benefits, stopping being a tax payer etc.. So, there is nothing more important than getting that child/young person into remission to prevent all/some of the above and keep the family as a functioning, loving unit.

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

All of the points in the previous question apply here also. Methotrexate is widely used as the gold standard treatment and intra-articular steroid injections are also very effective. However, if standard DMARD therapy fails then having the option of biologics is essential by comparison to the potential damage which long term use of steroids, for example, would cause. As previously mentioned, the use of biologics has been completely life transforming for many children enabling them to simply get on with their lives normally. We agree with the BSPAR submission that no child failing on Methotrexate should be denied access to biologics.

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

I am not aware of anyone who feels differently to my answers above.

5. What do patients and/or carers 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

These treatments are generally given by sub-cut injection which can be difficult for the child/young person to come to terms with initially as no child 'likes' needles and sometimes parents find it hard to inject their child because they don't like hurting them. However, usually the benefits of feeling better help to encourage the child and the parent that this is necessary and after a period of getting used to it, it becomes a part of normal life. Distraction techniques and rewards can help.

It can be challenging for some families to travel the distance to the specialist centre to get this treatment initiated, but once established on therapy, shared care arrangements would be set up with a local unit and the drugs would be delivered by a home care delivery company.

Side effects of injecting tend to focus around injection site reactions which usually resolve.

Please list any concerns patients or carers have about current NHS treatments in England.

Please list any concerns patients or carers have about the treatment(s) being appraised.

Parents are often worried about their children having to take 'toxic' medication but once it has been explained to them in a way which they can understand, i.e. that not treating the disease would be worse than any reaction to the medication which can be identified swiftly and sorted, they are usually accepting that this is a necessary part of their child's care and when they see that their child is getting better and symptoms subsiding, their attitude to the medication changes. We have seen this first hand with many families.

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

6. Patient population

Are there any groups of patients who might benefit more from the treatment(s) than others? If so, please describe them and explain why.

All children with JIA who fail on Methotrexate should be given the option of moving on to the most appropriate biologic therapy.

Are there any groups of patients who 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

Is your organisation familiar with the published research literature for the treatment(s)?

☐ Yes to a degree as NRAS provides the lay input to the BPSAR Register ☐ No

If you answered 'no', please skip the rest of section 7 and move on to section 8.

Please comment on whether patients' experience of using the treatment(s) as part of their routine NHS care reflects the experiences of patients in the clinical trials.

I can't answer this accurately in regard to children, but from experience in the adult world, there is often a difference in profile between patients in clinical trials and those seen in routine clinical care.

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?

We would support the BSPAR submission

If already available in the NHS, are there any side effects associated with treatment(s) being appraised that were not apparent in the clinical trials but have emerged during routine NHS care?

We would support the BSPAR submission

Are you aware of any relevant research on patient or carer views of the condition or existing treatments (for example, qualitative studies, surveys and polls)?

☐ Yes ☐ No

If yes, please provide references to the relevant studies.

8. Equality

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Protected characteristics are: age; being or becoming a transsexual person; being married or in a civil partnership; being pregnant or having a child; disability; race including colour, nationality, ethnic or national origin; religion, belief or lack of religion/belief; sex; sexual orientation.

Please let us know if you think that recommendations from this appraisal could have an adverse impact on any particular groups of people, such as:

- excluding from full consideration any people protected by the equality legislation who fall within the patient population for which the treatment is/will be licensed;
- having 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 treatment;
- any adverse impact on people with a particular disability or disabilities.

Please let us know if you think that there are any potential equality issues that should be considered in this appraisal.

Are there groups of patients who would have difficulties using the treatment(s) being appraised or currently available treatments? Please tell us what evidence you think would help the Committee to identify and consider such impacts.

I imagine that there might be issues in relation to compliance with treatment in families that have a record of alcohol or drug abuse where the care of the children may be in question. But I assume that this would be flagged and addressed by the treating unit working with local social care organisations. I also imagine that there may be issues in regard to ethnic groups where understanding English is a problem and culture is quite different.

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.)

Use of biologics in children and young people is still relatively new and the various registers based in Manchester are continuing to collect data but we have no real long term use data. However, there is no doubt that for children and young people failing on standard treatments, these therapies are transforming lives. We do need to maintain the focus on long term data collection.

Are there any other issues 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 submission.

- These therapies are an essential part of the treatment pathway for children and young people with JIA and they should have access to them
- They should also continue to have access to them if they take their disease into adulthood as this can sometimes be problematic with clinicians having to change the 'diagnosis' to RA in order to allow continuation of treatment.
- They can be life transforming for those who have failed on standard treatments, however, the research agenda must continue as their remains unmet need as they do not work for everyone.
- They have a major positive impact on reducing symptoms allowing the child and the family to return to and maintain normal life and day to day activities
- We must continue to collect long term data on their use
- They can significantly reduce disability