National Rheumatoid Arthritis Society

[redacted]
National Institute for Health and Clinical Excellence
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24th August 2011

Dear [redacted]

Tocilizumab for SJIA – NICE Minded negative decision

Following [redacted] submission of 24th March and the recent minded "no" decision by NICE I wish to make a further submission in regard to the STA in respect of Toclizumab for the treatment of juvenile idiopathic arthritis. We at NRAS are very concerned of the impact that denial of such an effective treatment for children with Systemic JIA will have on not just the child themselves but on their parents and siblings.

The scale of the problem

As previously stated in my submission in March the number of children that may be eligible for treatment with Tocilizumab is very small indeed. The number of children with Systemic JIA is approximately 1,200 and Tocilizumab would not necessarily be considered necessary or appropriate treatment for all of them therefore the number of children possibly being denied this life changing drug are in fact very few .Taking this into account if the "minded no" that the appraisal committee has given is based on cost grounds then I would draw their attention to the fact that the actual cost implications for the NHS would be relatively minimal. Clinicians that NRAS work with feel very strongly that there is a very strong evidence base to support the use of tocilizumab in sJIA and they will continue to try and access it for patients but this will be much more complex without NICE approval and will almost certainly mean some children do not access a drug that has potentially massive benefit.

The Impact to Children and their Families

I have heard and seen, having met families who are affected by JIA, the far reaching impact that having a child with such a long term condition. The impact on the child themselves can be not being able to fully participate in all aspects of family life such as family holidays, sports and fun activities to the impact on their education. School absences result in missed classes and can naturally impact on state examination results leading to reduced third level education and career prospects. The impact on the family as a whole the worry of a parent for their child's future, devastation of watching your child in pain and feeling of helplessness when unable to access treatments that have been proven to be effective. Siblings' family life can also be damaged if first consideration has to always be to the child with limited mobility and the requirement to always adapt family outings; holidays; day to day life around the JIA.

[redacted] who accompanied [redacted] to the NICE appraisal puts her thoughts forward in Appendix which is attached with this letter.

The Need for Tocilizumab
TNF blockade has been disappointing in its effects in methotrexate resistant children and adolescents, therefore given the severity of disease in Systemic JIA and a higher mortality rate, having access to a biologic with a different mode of action, i.e. Tocilizumab, is vital. Clinical Trials in Japan have demonstrated that many of the symptoms of SJIA can be controlled with periodic infusions of Tocilizumab and we await the publication of current UK trials for this agent in SJIA (TENDER) and in polyarticular JIA (CHERISH).

Potential Economic Benefits of using Tocilizumab vs standard (failing) treatment
The cost to individuals and their families of SJIA is high and should not be measured in monitory terms only. Giving a child with S JIA access to a drug that could open up a world of possibilities and potential will not only benefit the individual but also those who care for them as well as society and the economy.

The societal costs and savings if SJIA enters remission before causing damage allowing children to become adults better able to enter the workplace are considerable. There is the probability of being able to reduce other medication, importantly steroids, which are extremely damaging taken long term.

The costs of caring for children with JIA can also be considerable, especially when parents have to leave work or reduce their working hours to look after the child and the financial cost of reduced income simply adds to the overall burden of stress on the whole family.

It is abundantly clear to see the enormous potential in returning an extremely sick child, requiring significant on-going and expensive NHS services, to ‘normal’ health as demonstrated in Kieran’s story which was submitted with NRAS’s March submission. Steroid burden and toxicity can be huge in SJIA, and there is considerable evidence now available that disease control with Tocilizimab will allow steroid reduction potentially to zero.

I hope that the Committee will reconsider their “minded no” and NRAS are happy to be re-contacted for any further information as required.

Yours sincerely

[redacted]
[redacted] [redacted] [redacted] [redacted], acting on behalf of [redacted] [redacted], [redacted], in her absence.