



ARTHRTIS CARE CONSULTATION SUBMISSION

NICE Single Technology Appraisal:

Tocilizumab for the treatment of systemic juvenile idiopathic arthritis

April 2011

Arthritis and Arthritis Care

1. Arthritis is the biggest cause of physical disability in the UK, affecting up to 10 million people, including 12,000 children, and accounting for 30% of GP visits. It carries a huge economic as well as human and social cost, estimated at £7 billion annually in terms of lost labour in 2007.
2. Arthritis Care is the UK's leading organisation working with and for people with all forms of arthritis. We offer people with arthritis the information and support they need to make informed choices about managing their arthritis, to reach their potential in society and to fully participate in their communities.
3. We believe that people with arthritis are entitled to receive the best available treatment and medication, and to have their voice heard in decisions affecting their health – as enshrined in the NHS Constitution.

Juvenile idiopathic arthritis

4. JIA is a disease affecting 1 in 1,000 schoolchildren under the age of 16 years. 12,000 children in the UK have juvenile idiopathic arthritis.¹
5. The severity of the arthritis experienced by children with JIA can vary from mild to extremely disabling. Depending on which joints are affected, difficulties can be experienced in all areas of their lives. In young children physical development can be significantly delayed. Inflammation of the joints in JIA causes pain, swelling and muscle wasting, all of which can interfere with activities of daily life. Ongoing inflammation can lead to permanent joint damage and growth disturbance (for example, legs of unequal length).

¹ Silman AJ, Hochberg MC. Epidemiology of the Rheumatic Diseases. 2nd Ed. Oxford Medical Publications, (2001)

6. There are several different types of JIA, of which systemic JIA represents approximately 10% of the total. This type can have manifestations with systemic features, including temperature, rash and general malaise. Arthritis can be present and is often not an initial feature. Few drugs are effective on this disease: methotrexate can be effective in some children.
7. The most common age for systemic JIA to commence is before five years. It is the least common form of juvenile arthritis, affecting less than 1 in 10 children with arthritis. It used to be known as Still's disease.
8. Approximately 50% of patients with the systemic JIA will still have the active disease 15 years after onset. This group will continue to have considerable disability.

General observations on the preliminary findings

9. Arthritis Care described NICE's decision, in July 2010, to approve tocilizumab for people with moderate to severe RA for whom other treatments have not worked as an "important breakthrough", while expressing regret that tocilizumab would still only be available as "a last resort", thereby giving clinicians little leeway in prescribing it.
10. Arthritis Care notes that etanercept is currently recommended for Poly articular pattern in all types of JIA (TA 35). It is not effective in control of systemic symptoms in systemic type JIA. Tocilizumab has been shown to be an effective treatment for the systemic symptoms of JIA. It may also have an effect on the arthritis. It therefore has additional benefit above anti - TNF inhibitors (etanercept & adalimumab). Arthritis Care therefore would recommend consideration of approval of tocilizumab as a welcome addition to the existing anti - TNF drugs in JIA.

Cost-effectiveness

11. It is important to take a broad view of the costs involved, beyond the financial costs to secondary care. The NAO has clearly highlighted that non-biological treatment of rheumatoid arthritis (RA) carries significant costs to primary and secondary care, in addition to the person with RA. On the other hand, recent evidence compiled by the NAO shows that biological treatment of RA saves money, e.g. in terms of reduced emergency admissions and less reliance on the health sector generally.
12. The NAO has also developed an economic model in connection to its aforementioned report on services for people with RA. This model states that the analyses conducted "have provided clear evidence that better value for money could be achieved by providing more rapid treatment for people with early onset rheumatoid arthritis," improving patients' quality of life and delivering productivity gains for the economy. It is possible that the same cost effectiveness in early treatment could well be found in the case of JIA.
13. The document goes on to say that "although it could increase the cost to the NHS in the short-term, it would be cost effective, and could be cost saving in the longer-term". Finally, it states that "the analyses also confirm the NICE

conclusion that intensive early treatment with step-down strategy is more cost effective than current routine practice in terms of sequential DMARD treatment (which is dominated by mono switch treatment strategy), and suggest that potential cost savings to the NHS could be realised in the medium to long-term.”

Conclusion

14. Arthritis Care feels that any cost-effectiveness analyses of new medications, including tocilizumab, should take strongly into account patient experience as fundamental and essential drivers of decisions regarding people’s health.
15. Crucially, being able to access the best treatments which work for each individual person with JIA is essential, especially when an anti-TNF has proved to be ineffective. Arthritis Care believes that tocilizumab represents good effective alternative treatment and value for money in the treatment of systemic JIA..

Appendix A

Arthritic Care testimony: a parent of a child with systemic JIA

‘There are definitely times when we plan everything around my daughter and how she is feeling. Her younger twin sisters have adapted to her having arthritis and will fetch and carry things for her. But she is treated no differently from the others — she still gets chores. Of course, me being in hospital with her for weeks and weeks at a time affected everyone in the family.

The best way I have found to support my daughter is to really encourage her — I never say no to things she wants to do. She is determined not to let arthritis rule her life and will know herself if she is doing too much. She used to be out all day and then in pain the next, but since her early teens she has learnt to pace herself.

She’s 18 soon and can do everything for herself now — she still wants me at hospital appointments but I don’t say anything unless I’m asked.’