NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Single Technology Appraisal

Abatacept for the treatment of juvenile idiopathic arthritis

Final scope

Remit/appraisal objective

To appraise the clinical and cost effectiveness of abatacept within its licensed indication for the treatment of juvenile idiopathic arthritis.

Background

Juvenile idiopathic arthritis (JIA) describes a clinically heterogeneous group of syndromes that begin before 16 years of age and last for more than six weeks. JIA is characterised by persistent joint swelling, pain and limitation of movement. The clinical signs and symptoms of JIA differ between groups of patients. The cause of JIA is poorly understood, but may relate to genetic and environmental factors.

There are seven categories of JIA: systemic, oligo arthritis (formerly pauciarticular), polyarthritis rheumatoid factor positive, polyarthritis rheumatoid factor negative, enthesitis related arthritis, psoriatic arthritis and unclassified (types that do not correspond to any, or to more than one, category). There are approximately 1000 new cases of JIA a year; in Europe approximately 50% of these are pauciarticular, 25% are polyarticular and 10% are systemic onset types.

JIA can lead to growth retardation, joint contractures, eye problems, destructive joint disease requiring joint replacements, and permanent disability. JIA can impair children's personal and social functioning and development. Children often miss out on schooling and normal childhood activities, and as adults they may be limited in, or unable to, work. It may also have a considerable impact upon the family of the child.

Standard treatment for JIA includes the use of the disease modifying anti rheumatic drugs (DMARDs), usually methotrexate, alongside intra-articular and systemic corticosteroids and non steroidal anti-inflammatory drugs (NSAIDS). NICE has issued guidance on the use of etanercept (a tumour necrosis factor inhibitor) for the treatment of polyarticular JIA. Etanercept is recommended for the treatment of children aged 4 to 17 years who have active polyarticular JIA in at least five joints and whose condition has not responded adequately to methotrexate or who have been unable to tolerate treatment with methotrexate. It is estimated that each year 200 children with JIA will start treatment with TNF inhibitors following the failure of methotrexate treatment.

The technology

Abatacept (Orencia, Bristol Myers Squibb) is a selective co-stimulation modulator which prevents T-cell activation by binding to CD80 and CD86 and inhibiting a co-stimulating signal by binding to B7 (CD80), a receptor found on antigen-presenting cells. Abatacept is administered by intravenous infusion on days 1, 15 and 29 of treatment and then every 28 days. Abatacept does not currently have a marketing authorisation for the treatment of juvenile idiopathic arthritis and there is no information currently available about the proposed indication for abatacept. A phase III clinical trial has investigated the use of abatacept following the failure of conventional DMARDs in patients with the following subtypes of JIA: extended oligoarthritis, polyarthritis (both rheumatoid factor positive and negative) and systemic-onset polyarticular.

Intervention(s)	Abatacept
Population(s)	Children and adolescents with JIA
Standard comparators	Management strategies involving DMARDs without abatacept, including treatment with • conventional DMARDs • biologic DMARDs including etanercept, adalimumab and infliximab
Outcomes	The outcome measures to be considered include: • physical function • disease activity • joint damage • pain • adverse effects of treatment • health-related quality of life • mortality
Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. The time horizon for the economic evaluation will be based on the time period over which costs and benefits can reasonably be expected to be experienced. The time horizon will reflect the chronic nature of JIA. Costs will be considered from an NHS and Personal Social Services perspective.

Other considerations	If the evidence allows, the appraisal will attempt to identify criteria for selecting patients for whom this treatment would be particularly appropriate, and the stage in the pathway of care when this technology should be used. If the evidence allows, the appraisal should consider the benefits and costs associated with avoiding long term disability and joint replacement. If the evidence allows, subject to marketing authorisation the appraisal should consider children with both juvenile idiopathic arthritis and uveitis. Guidance will only be issued in accordance with the marketing authorisation.
Related NICE recommendations	Related Technology Appraisals: NICE Technology Appraisal No.35, The use of etanercept for the treatment of juvenile idiopathic arthritis, 2002. Technology Appraisal in Preparation, Tocilizumab for the treatment of juvenile idiopathic arthritis.