NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Canakinumab for treating systemic juvenile idiopathic arthritis

Updated draft scope (pre-invitation)

Draft remit/appraisal objective

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

Background

Juvenile idiopathic arthritis (JIA) is a term that covers a heterogeneous group of syndromes in which the onset of inflammatory arthritis occurs before the age of 16 years and lasts for more than 6 weeks. The cause of JIA is poorly understood, but may relate to genetic and environmental factors. Systemic JIA is a multi-organ disease characterised by arthritis symptoms (persistent joint swelling, pain and limitation of movement), intermittent fever, transient rash, liver and spleen enlargement.

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

JIA is a relatively rare disease, with an estimated incidence in the UK of 0.1 per 1000 children per year, equivalent to 1000 children diagnosed per year. The prevalence is in the order of 1 per 1000 children, and about 10,000 children in the UK are affected. Approximately 10% of children diagnosed with JIA have systemic JIA.

Treatment aims to control pain, fever and inflammation, and reduce joint damage, disability and loss of function, thereby improving quality of life. The standard treatment for systemic JIA includes combinations of non-steroidal anti-inflammatory drugs (NSAIDs), analgesics, corticosteroids. Oral corticosteroids such as prednisone are used initially before progressing to non-biological disease modifying anti-rheumatic drugs (DMARDs) such as methotrexate. If symptoms are not adequately controlled with methotrexate, anakinra or tocilizumab are used when considered necessary. Tocilizumab is currently the only biologic DMARD licensed in the UK for the treatment of active systemic JIA in children and young people. NICE has recommended tocilizumab for the treatment of systemic JIA in children and young people

whose disease has responded inadequately to NSAIDs, systemic corticosteroids and methotrexate (NICE technology appraisal No. 238). Further treatment options that may be considered include non-drug therapies such as surgery and physical therapy.

The technology

Canakinumab (Ilaris, Novartis Pharmaceuticals UK) is a monoclonal antibody that inhibits the activity of cytokine interleukin-1 beta (IL-1 beta), which reduces inflammation and tissue destruction. Canakinumab is administered by subcutaneous injection.

Canakinumab does not currently have a UK marketing authorisation for the treatment of systemic JIA. It has been studied in clinical trials as a monotherapy or in combination with methotrexate compared with placebo in children and young people aged 2 to 19 years with active systemic JIA.

Intervention(s)	Canakinumab (with or without methotrexate)
Population(s)	• People aged 2 years and older with active systemic juvenile idiopathic arthritis whose disease has responded inadequately to previous therapy with NSAIDs and systemic corticosteroids. • People aged 2 years and older with active systemic juvenile idiopathic arthritis whose disease has responded inadequately to previous therapy with NSAIDs, systemic corticosteroids and methotrexate.
Comparators	For people aged 2 years and older with active systemic juvenile idiopathic arthritis whose disease has responded inadequately to previous therapy with NSAIDs and systemic corticosteroids: • methotrexate For people aged 2 years and older with active systemic juvenile arthritis whose disease has responded inadequately to previous therapy with NSAIDs, systemic corticosteroids and methotrexate: • anakinra • tocilizumab
Outcomes	The outcome measures to be considered include: • disease activity • physical function

	• joint damage • pain • fever • corticosteroid sparing • mortality • adverse effects of treatment • health-related quality of life
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 reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared. Costs will be considered from an NHS and Personal Social Services perspective. The availability of any patient access schemes for the intervention or comparator technologies should be taken into account.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation.
Related NICE recommendations and NICE pathways	Related Technology Appraisals: Technology Appraisal No.238, December 2011, 'Tocilizumab for the treatment of systemic juvenile idiopathic arthritis'. Expected review date December 2014. Suspended technology appraisal, 'Abatacept for the treatment of juvenile idiopathic arthritis'.
Related NHS England policy	Service specification for paediatric medicine rheumatology http://www.england.nhs.uk/wp-content/uploads/2013/06/e03-paedi-medi-rheum.pdf

Questions for consultation

Have the most appropriate comparators for canakinumab for treating active systemic juvenile idiopathic arthritis been included in the scope?

- Are the comparators listed routinely used in clinical practice?

Are there any subgroups of people in whom the technology is expected to be more clinically effective and cost effective or other groups that should be examined separately?

Please consider whether in the remit or the scope there are any issues relevant to equality. Please pay particular attention to whether changes need to be made to the remit or scope in order to promote equality, eliminate unlawful discrimination, or foster good relations between people who share a characteristic protected by the equalities legislation and those who do not share it, or if there is information that could be collected during the assessment process which would enable NICE to take account of equalities issues when developing guidance.

Do you consider the technology to be innovative in its potential to make a significant and substantial impact on health-related benefits and how it might improve the way that current need is met (is this a 'step-change' in the management of the condition)?

Do you consider that the use of the technology can result in any potential significant and substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the Appraisal Committee to take account of these benefits.

NICE intends to appraise this technology through its Single Technology Appraisal (STA) Process. We welcome comments on the appropriateness of appraising this topic through this process. (Information on the Institute's Technology Appraisal processes is available at http://www.nice.org.uk/aboutnice/howwework/devnicetech/technologyappraisalprocessguides/technology_appraisal_process_guides.jsp)