NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Baricitinib for treating juvenile idiopathic arthritis in children and young people aged 1 to 17

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of baricitinib within its marketing authorisation for treating juvenile idiopathic arthritis in children and young people aged 1 to 17.

Background

Juvenile idiopathic arthritis (JIA) is a group of chronic inflammatory diseases. It is characterised by joint inflammation which starts before the age of 16 and lasts for more than 6 weeks. JIA causes joint pain, joint swelling and limitation of movement. Symptoms vary between people and can change from day to day. When the condition becomes more active and the symptoms worsen, this is known as a 'flare'. In more severe cases, JIA can cause growth retardation, joint contractures, joint disease requiring joint replacements, eye problems, other extra-articular manifestations (such as inflammatory bowel disease and psoriasis) and permanent disability.

JIA can impair personal and social functioning as well as development. Children often miss out on schooling and other childhood activities, and as adults they may be limited in their ability to work. JIA may also have a considerable impact on the family of the child, including parents and carers who may need to miss work to take children to appointments. About 50% of children with JIA will not achieve remission from the condition, despite treatment, and will need further rheumatological care as adults.[1]

JIA has an annual incidence of 1 per 10,000 children in the UK[2] (equivalent to around 1,400 children diagnosed per year[3]).Its overall prevalence is 1 in 1000 children[2] and it is estimated that around 14,000, children and young people have JIA in the UK based on ONS 2021 mid-year UK population estimates.[3,4] There are several different types of JIA, of which oligoarthritis (affecting 4 or fewer joints during the first 6 months of disease) and polyarthritis (affecting 5 or more joints during the first 6 months of the disease) are the most common.

Treatment aims to control joint pain and inflammation; reduce joint damage, disability and loss of function; and maintain or improve quality of life. Following diagnosis, people are offered corticosteroids (administered intravenously or intra-articular). If active inflammation remains, methotrexate is offered, which is a conventional disease-modifying anti-rheumatic drug (DMARD). If there is no improvement in symptoms, a biologic DMARD is added. It is estimated that up to a third of all children who start methotrexate need to progress to a biologic.[2]

NICE has recommended several biologic DMARDs as options for treating JIA. These include tofacitinib (technology appraisal guidance 735), tocilizumab (technology appraisal guidance 238) and abatacept, adalimumab, etanercept and tocilizumab (technology appraisal guidance 373). NHS England's Clinical Commissioning Policy

Statement outlines that other biologic therapies may be used in clinical practice including infliximab, anakinra and rituximab.[2] Eligibility and treatment choice are based on JIA subtype, age and previous lines of therapy.

The technology

Baricitinib (Olumiant, Eli Lilly & Company Limited) does not currently have a marketing authorisation in the UK for the treatment of JIA. It has been studied in clinical trials compared with placebo in people with JIA aged from 1 to 17 years. Participants must have had an inadequate response to at least one conventional or biologic DMARD.

Baricitinib has a UK marketing authorisation for treating another rheumatic disease, moderate to severe rheumatoid arthritis in adult patients who have responded inadequately to, or who are intolerant to one or more DMARDs.

Intervention(s)	Baricitinib
Population(s)	Children and young people aged 1 to 17
Subgroups	If the evidence allows the following subgroups will be considered: • JIA sub-types (e.g. polyarticular, extended oligoarticular) • Levels of disease activity
Comparators	• methotrexate • tofacitinib • abatacept • adalimumab • etanercept • tocilizumab • infliximab • rituximab (rheumatoid factor-positive arthritis) • anakinra (systemic JIA)

Outcomes	The outcome measures to be considered include: • disease activity (including disease flares and remission) • physical function • joint damage • body weight and height • pain • corticosteroid sparing • JIA subgroup-specific outcomes where relevant (e.g. enthesitis and dactylitis counts) • adverse effects of treatment • health-related quality of life (of patients and carers)
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. If the technology is likely to provide similar or greater health benefits at similar or lower cost than technologies recommended in published NICE technology appraisal guidance for the same indication, a cost comparison may be carried out. 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 commercial arrangements for the intervention, comparator and subsequent treatment technologies will be taken into account'. The availability and cost of biosimilar and generic products should be taken into account.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.
Related NICE recommendations	**Related Technology Appraisals:** Tofacitinib for treating juvenile idiopathic arthritis (2021). NICE Technology Appraisal 735.

	Abatacept, adalimumab, etanercept and tocilizumab for treating juvenile idiopathic arthritis (2015). NICE Technology Appraisal 373. Tocilizumab for the treatment of systemic juvenile idiopathic arthritis (2011). NICE Technology Appraisal 238. Anakinra for treating Still's disease (2021) NICE Technology Appraisal 685 **Related technology appraisals in development:** Sarilumab for treating polyarticular or oligoarticular juvenile idiopathic arthritis in people 2 to 17 years [TS ID 10237] **Terminated appraisals:** 'Canakinumab for treating systemic juvenile idiopathic arthritis' (terminated appraisal) (2013). NICE Technology Appraisal 302.
Related National Policy	NHS England (2015) Clinical Commissioning Policy Statement: Biologic Therapies for the treatment of Juvenile Idiopathic Arthritis (JIA) The NHS Long Term Plan, 2019. NHS Long Term Plan NHS England (2018/2019) NHS manual for prescribed specialist services (2018/2019) Section 138. Department of Health and Social Care, NHS Outcomes Framework 2016-2017: Domains 2 to 5. https://www.gov.uk/government/publications/nhs-outcomes-framework-2016-to-2017

Questions for consultation

Which treatments are currently used for JIA in the NHS?

Where do you consider baricitinib will fit into the existing care pathway for JIA?

Which subtypes of JIA do you anticipate that baricitinib would be used for?

Would extra-articular manifestations be useful outcomes to include, if so which ones?

Would baricitinib be a candidate for managed access?

Do you consider that the use of baricitinib can result in any potential 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 committee to take account of these benefits.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which baricitinib will be licensed;
- could lead to recommendations that have 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 technology;
- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

NICE is considering evaluating this technology through its cost comparison evaluation process.
Please provide comments on the appropriateness of appraising this topic through this process.
(Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation).

Technologies can be evaluated through the cost-comparison process if they are expected to provide similar or greater health benefits, at a similar or lower cost, compared with technologies that have been previously recommended (as an option) in published NICE guidance for the same indication. Companies can propose cost-comparison topics to NICE at any stage during topic selection and scoping. NICE will route technologies for evaluation through the cost-comparison process if it is agreed during scoping that the process is an appropriate route to establish the clinical and cost effectiveness of the technology.

NICE's health technology evaluations: the manual states the methods to be used where a cost comparison case is made.

- Is the technology likely to be similar in its clinical effectiveness and resource use to any of the comparators? Or in what way is it different to the comparators?
- Will the intervention be used in the same place in the treatment pathway as the comparator(s)? Have there been any major changes to the treatment pathway recently? If so, please describe.

- Will the intervention be used to treat the same population as the comparator(s)?
- Overall is the technology likely to offer similar or improved health benefits compared with the comparators?
- Would it be appropriate to use the cost-comparison methodology for this topic?

References

1 Minden K, Kiessling U, Listing J, et al. (2000) Prognosis of patients with juvenile chronic arthritis and juvenile spondyloarthropathy. Journal of Rheumatology 27:2256–63

2 NHS England (2015) Clinical Commissioning Policy Statement: Biologic Therapies for the treatment of Juvenile Idiopathic Arthritis (JIA). Accessed June 2023

3 Office for National Statistics. National population projections: Mid 2021-based. Prevalence rate applied to 0-17 UK population Accessed July 2023

4 National Rheumatoid Arthritis Society. Juvenile idiopathic arthritis (JIA) https://nras.org.uk/resource/juvenile-idiopathic-arthritis Accessed June 2023