GIG CYMRU NHS WALES

Pwyllgor Gwasanaethau Iechyd Arbenigol Cymru (PGIAC)

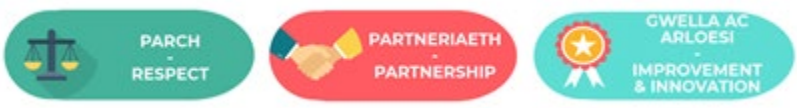
Welsh Health Specialised Services Committee (WHSSC)

Specialised Services Policy Position PP229

Tofacitinib for treating juvenile idiopathic arthritis in people from 2 years old up to their to 16th birthday

May 2022

Version 1.0



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Target audience	Chief Executives, Medical Directors, Directors of Finance, Directors of Nursing, Chief Pharmacists
Description	NHS Wales routinely commission this specialised service in accordance with the criteria described in this policy
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Contents

Policy Statement

Welsh Health Specialised Services Committee (WHSSC) commission Tofacitinib for people aged from 2 years up to their 16th birthday with treating juvenile idiopathic arthritis in accordance with the criteria outlined in this document.

In creating this document WHSSC has reviewed the relevant guidance issued by National Institute of Health and Care Excellence (NICE)[1] and has concluded that Tofacitinib should be made available.

Disclaimer

WHSSC assumes that healthcare professionals will use their clinical judgment, knowledge and expertise when deciding whether it is appropriate to apply this policy position statement.

This policy may not be clinically appropriate for use in all situations and does not override the responsibility of healthcare professionals to make decisions appropriate to the circumstances of the individual patient, in consultation with the patient and/or their carer or guardian, or Local Authority.

WHSSC disclaims any responsibility for damages arising out of the use or non-use of this policy position statement.

[1] Overview | Tofacitinib for treating juvenile idiopathic arthritis | Guidance | NICE

1. Introduction

This Policy Position Statement has been developed for the planning and delivery of Tofacitinib for people with juvenile idiopathic arthritis aged from 2 years up to their 16th birthday who are resident in Wales. This service will only be commissioned by the Welsh Health Specialised Services Committee (WHSSC) and applies to residents of all seven Health Boards in Wales.

1.1 Plain language summary

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. It is characterised by relapsing and remitting episodes of inflammation of the synovial membrane of the joints (synovitis) which, unless treated, leads to damage and deformity of the affected joints and subsequent disability.

A number of subtypes of JIA exist including:

- Oligoarthritis is when fewer than 5 joints are affected in the first six months. Children may go on to develop arthritis in other joints after the first 6 months and this will then be called extended oligoarthritis.
- Polyarthritis is diagnosed when 5 or more joints are affected in the first 6 months. There are 2 types of polyarthritis, one where a blood test is positive for rheumatoid factor (RF) and one where it is negative.
- Enthesitis Related Arthritis (ERA) is when there is inflammation in the places where the tendons attach to the bone and a child may suffer from pain in the bottoms of their feet, around their hips, knees or in their back.
- Psoriatic Arthritis can affect any joint but often involves the fingers and toes. There may have been a swollen, 'sausage' shaped toe, when the child was younger that got better on its own; this is known as dactylitis. Psoriasis is a scaly rash typically affecting the knees and elbows. Often, psoriatic arthritis is diagnosed when there is no evidence of psoriasis in the child but there is a family member with psoriasis and some typical changes to the nails.
- Systemic onset JIA is diagnosed if your child had a fever or rash at the beginning of their illness.

Tofacitinib, also known as Xeljanz, is a type of drug known as a Janus kinase (JAK) inhibitor. It works by blocking the action of Janus kinase enzymes, which are involved in inflammation[2].

1.2 Aims and Objectives

This Policy Position Statement aims to define the commissioning position of WHSSC on the use of Tofacitinib for people a with juvenile idiopathic arthritis (JIA) aged from 2 years up to their 16th birthday.

The objectives of this policy are to:

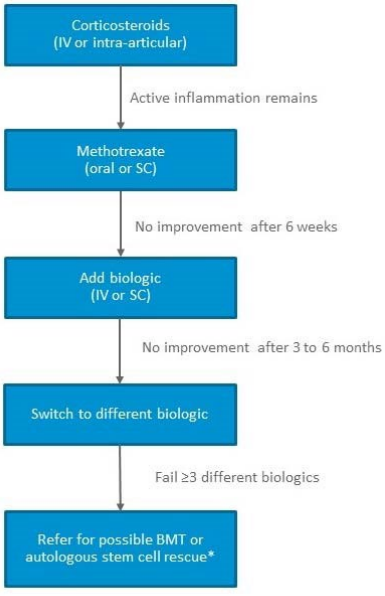
- ensure commissioning for the use of Tofacitinib is evidence based
- ensure equitable access to Tofacitinib
- define criteria for people with JIA to access treatment
- improve outcomes for people with JIA

1.3 Epidemiology

JIA is the most common chronic rheumatic disease in children. Its estimated annual incidence in the UK is 1 in 10,000. This equates to 63 cases per year in Wales (based on mid-2019 population estimates). Its estimated annual prevalence in the UK is 1 in 1,000 which equates to 630 children in Wales with JIA. Overall, JIA is more common in girls than in boys[3].

1.4 Current Treatment and pathway

The current treatment pathway for JIA is shown in Figure 1 below:



*Not a relevant treatment option in Polyarticular course juvenile idiopathic arthritis (pcJIA)
BMT= bone marrow transplant; IV=intravenous; SC=subcutaneous

[2] https://www.nice.org.uk/guidance/ta735/documents/final-scope
[3] https://www.nice.org.uk/guidance/gid-ta10706/documents/committee-papers

Following diagnosis, patients are first given steroids. If active inflammation remains, methotrexate is used; this can be oral or SC, although SC is advised before escalation to biologic treatment. If there is no improvement in symptoms with methotrexate treatment, a biologic disease-modifying anti-rheumatic drug (bDMARD; either adalimumab, etanercept, tocilizumab or abatacept) is added. Patients who have not responded to treatment after 3-6 months are then switched to a different biologic. If patients have still not responded after trying at least three different biologics, they may be referred for bone marrow transplant or autologous stem cell rescue, although this is rare in polyarticular course JIA.

Currently approved biologic DMARDs for JIA belong to different drug classes: etanercept and adalimumab are TNF inhibitors, abatacept is a T-cell co-stimulatory modulator and tocilizumab is an IL-6 inhibitor. The best choice of biologic to use after failure of the first biologic remains unclear.

Clinical experts consulted during the development of NICE guidance TA373 stated that they consider the available biologics to be similar in terms of effectiveness and that their choice of biologic takes into account patient preference, patient characteristics and previous treatments[4].

1.5 Proposed Treatment

Tofacitinib is a Janus kinase (JAK) inhibitor. It preferentially inhibits signalling by cytokine receptors that associate with JAK3 and/or JAK1. Inhibition of JAK disrupts signalling pathways that are critical to immune and inflammatory responses.

Treatment with tofacitinib will be prescribed by the paediatric rheumatology team at the tertiary centre.

1.6 What NHS Wales has decided

WHSSC has carefully reviewed the relevant guidance issued by National Institute of Health and Care Excellence (NICE). We have concluded that Tofacitinib should be made available within the criteria set out in section 2.1.

[4] https://www.nice.org.uk/guidance/gid-ta10706/documents/committee-papers

2. Criteria for Commissioning

The Welsh Health Specialised Services Committee approve funding of Tofacitinib for people with JIA aged from 2 years up to their 16th birthday, in-line with published NICE guidance[5] and the criteria identified in this policy.

2.1 Criteria

- Tofacitinib is recommended as an option for treating active polyarticular juvenile idiopathic arthritis (rheumatoid factor positive or negative polyarthritis and extended oligoarthritis), and juvenile psoriatic arthritis in people 2 years and older[6]. This is if their condition has responded inadequately to previous treatment with disease-modifying antirheumatic drugs, and only if:
    - a tumour necrosis factor (TNF)-alpha inhibitor is not suitable or does not control the condition well enough, and
    - the company provides tofacitinib according to the commercial arrangement
- Tofacitinib can be used with methotrexate, or as monotherapy when methotrexate is not tolerated or if continued treatment with methotrexate is inappropriate.
- If tofacitinib is one of a range of treatments considered suitable by patients, or their parents or carers, and their clinicians, choose the least expensive (taking into account administration costs and commercial arrangements).
- This recommendation is not intended to affect treatment with tofacitinib that was started in the NHS before this guidance was published. Children and young people having treatment outside these recommendations may continue without change to the funding arrangements in place for them before this guidance was published, until they and their NHS clinician consider it appropriate to stop. This decision should be made jointly by the clinician, the child or young person, and their parents or carers.

2.2 Continuation of Treatment

Healthcare professionals are expected to review a patient's health at regular intervals to ensure they are demonstrating an improvement to their health due to the treatment being given.

[5] Overview | Tofacitinib for treating juvenile idiopathic arthritis | Guidance | NICE

[6] This treatment is commissioned by WHSSC for patients aged from 2 years up to their 16th birthday

If no improvement to a patient's health has been recorded then clinical judgement on the continuation of treatment must be made by the treating healthcare professional.

2.3 Acceptance Criteria

The service outlined in this specification is for patients ordinarily resident in Wales, or otherwise the commissioning responsibility of the NHS in Wales. This excludes patients who whilst resident in Wales, are registered with a GP practice in England, but includes patients resident in England who are registered with a GP Practice in Wales.

2.4 Exceptions

If the patient does not meet the criteria for treatment as outlined in this policy, an Individual Patient Funding Request (IPFR) can be submitted for consideration in line with the All Wales Policy: Making Decisions on Individual Patient Funding Requests. The request will then be considered by the All Wales IPFR Panel.

If the patient wishes to be referred to a provider outside of the agreed pathway, and IPFR should be submitted.

Further information on making IPFR requests can be found at: Welsh Health Specialised Services Committee (WHSSC) | Individual Patient Funding Requests

2.5 Transition Arrangements

Transition arrangements should be in line with Transition from children's to adults' services for young people using health or social care services NICE guidance NG43 and the Welsh Government Transition and Handover Guidance.

Transition involves a process of preparation for young people and their families for their transition to adulthood and their transition to adult services. This preparation should start from early adolescence 12-13 year olds. The exact timing of this will ideally be dependent on the wishes of the young person but will need to comply with local resources and arrangements.

The transition process should be a flexible and collaborative process involving the young person and their family as appropriate and the service.

The manner in which this process is managed will vary on an individual case basis with multidisciplinary input often required and patient and family choice taken into account together with individual health board and environmental circumstances factored in.

2.6 Blueteq and reimbursement

Tofacitinib will only be funded for patients registered via the Blueteq system and where an appropriately constructed MDT has approved its use within highly specialised paediatric endocrinology centres.

Where the patient meet the criteria in this policy and the referral is received by an agreed centre, a Blueteq form should be completed for approval. For further information on accessing and completing the Blueteq form please contact WHSSC using the following e-mail address: WHSSC.blueteq@wales.nhs.uk

If a non-contracted provider wishes to treat a patient that meets the criteria they should contact WHSSC (e-mail: WHSSC.IPC@Wales.nhs.uk). They will be asked to demonstrate they have an appropriate MDT in place.

Funding is approved on the basis that tofacitinib is prescribed and administered in accordance with its marketing authorisation[7,8].

Tofacitinib licensed for treating juvenile idiopathic arthritis is available as a 1ml/ml oral solution and 5mg film coated tablets[2]. The cost per pack is £591.45 excluding VAT for the 240ml pack of oral solution and £690.03 excluding VAT per pack of 56x5mg film coated. The company has a commercial access agreements. This makes tofacitinib available to the NHS with a discount. The size of the discount is commercial in confidence. Health Boards in Wales should refer to the AWTTC Vault for further information on the Patient Access Scheme (PAS) price.

2.7 Clinical Outcome and Quality Measures

The Provider must work to written quality standards and provide monitoring information to the lead commissioner.

The centre must enable the patient's, carer's and advocate's informed participation and to be able to demonstrate this. Provision should be made for patients with communication difficulties and for children, teenagers and young adults.

2.8 Responsibilities

Referrers should:

- inform the patient that this treatment is not routinely funded outside the criteria in this policy, and
- refer via the agreed pathway.

[7] XELJANZ 1 mg/mL oral solution - Summary of Product Characteristics (SmPC) - (emc) (medicines.org.uk)
[8] XELJANZ 5 mg film-coated tablets - Summary of Product Characteristics (SmPC) - (emc) (medicines.org.uk)

Clinicians considering treatment should:

- discuss all the alternative treatment with the patient
- advise the patient of any side effects and risks of the potential treatment
- inform the patient that treatment is not routinely funded outside of the criteria in the policy, and
- confirm that there is contractual agreement with WHSSC for the treatment.

In all other circumstances an IPFR must be submitted.

2.9 Action to be taken

- Health Boards are to circulate this Policy Position Statement to all Hospitals/MDTs to inform them of the conditions under which the technology will be commissioned.
- Health Boards are to ensure that all providers are purchasing tofacitinib at the agreed discounted price.
- Health Boards are to ensure that all providers understand the need to approve tofacitinib at the appropriate MDT and are registering use on the Blueteq system, and the treatment will only be funded where the Blueteq minimum dataset is fully and accurately populated.
- Providers are to determine estimated patient numbers and the current dose of any patient(s) who will transfer from any company compassionate use scheme or EAMS.
- The Provider should work to written quality standards and provide monitoring information to WHSSC on request.

3. Documents which have informed this policy

The following documents have been used to inform this policy:

- **National Institute of Health and Care Excellence (NICE) guidance**
  - Tofacitinib for treating juvenile idiopathic arthritis, NICE Technology appraisal guidance (TA735). October 2021

This document should be read in conjunction with the following documents:

- **NHS Wales**
  - All Wales Policy: Making Decisions in Individual Patient Funding requests (IPFR).

4. Date of Review

This document will be reviewed when information is received which indicates that the policy requires revision.

5. Putting Things Right

5.1 Raising a Concern

Whilst every effort has been made to ensure that decisions made under this policy are robust and appropriate for the patient group, it is acknowledged that there may be occasions when the patient or their representative are not happy with decisions made or the treatment provided.

The patient or their representative should be guided by the clinician, or the member of NHS staff with whom the concern is raised, to the appropriate arrangements for management of their concern.

If a patient or their representative is unhappy with the care provided during the treatment or the clinical decision to withdraw treatment provided under this policy, the patient and/or their representative should be guided to the LHB for NHS Putting Things Right. For services provided outside NHS Wales the patient or their representative should be guided to the NHS Trust Concerns Procedure, with a copy of the concern being sent to WHSSC.

5.2 Individual Patient Funding Request (IPFR)

If the patient does not meet the criteria for treatment as outlined in this policy, an Individual Patient Funding Request (IPFR) can be submitted for consideration in line with the All Wales Policy: Making Decisions on Individual Patient Funding Requests. The request will then be considered by the All Wales IPFR Panel.

If an IPFR is declined by the Panel, a patient and/or their NHS clinician has the right to request information about how the decision was reached. If the patient and their NHS clinician feel the process has not been followed in accordance with this policy, arrangements can be made for an independent review of the process to be undertaken by the patient's Local Health Board. The ground for the review, which are detailed in the All Wales Policy: Making Decisions on Individual Patient Funding Requests (IPFR), must be clearly stated

If the patient wishes to be referred to a provider outside of the agreed pathway, an IPFR should be submitted.

Further information on making IPFR requests can be found at: Welsh Health Specialised Services Committee (WHSSC) | Individual Patient Funding Requests

6. Equality Impact and Assessment

The Equality Impact Assessment (EQIA) process has been developed to help promote fair and equal treatment in the delivery of health services. It aims to enable Welsh Health Specialised Services Committee to identify and eliminate detrimental treatment caused by the adverse impact of health service policies upon groups and individuals for reasons of race, gender re-assignment, disability, sex, sexual orientation, age, religion and belief, marriage and civil partnership, pregnancy and maternity and language (Welsh).

This policy has been subjected to an Equality Impact Assessment.

The Assessment demonstrates the policy is robust and there is no potential for discrimination or adverse impact. All opportunities to promote equality have been taken.

Annex i Codes

Code Category	Code	Description
ICD-10	M080	Juvenile idiopathic arthritis (JIA)

Annex ii Abbreviations and Glossary

Abbreviations

IPFR Individual Patient Funding Request

JIA Juvenile Idiopathic Arthiritis

WHSSC Welsh Health Specialised Services Committee

Glossary

Individual Patient Funding Request (IPFR)

An IPFR is a request to Welsh Health Specialised Services Committee (WHSSC) to fund an intervention, device or treatment for patients that fall outside the range of services and treatments routinely provided across Wales.

Welsh Health Specialised Services Committee (WHSSC)

WHSSC is a joint committee of the seven local health boards in Wales. The purpose of WHSSC is to ensure that the population of Wales has fair and equitable access to the full range of Specialised Services and Tertiary Services. WHSSC ensures that specialised services are commissioned from providers that have the appropriate experience and expertise. They ensure that these providers are able to provide a robust, high quality and sustainable services, which are safe for patients and are cost effective for NHS Wales.