GIG CYMRU NHS WALES
Cyd-bwyllgor Comisiynu
Joint Commissioning Committee

Commissioning Medicines for Children in Specialised Services

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Document name	Commissioning Medicines for Children in Specialised Services
Author	NHS Wales Joint Commissioning Committee
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Target audience	Chief Executives, Medical Directors, Directors of Finance Neurologists, Gastroenterologists, Endocrinologists, Rheumatologists, Consultant Paediatricians, Lead Pharmacists
Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy
Document No	CP299
Review Date	2028

GIG CYMRU NHS WALES | Cyd-bwyllgor Comisiynu Joint Commissioning Committee

Contents

Policy Statement

NHS Wales Joint Commissioning Committee (NWJCC) will commission treatments for patients aged less than 18 years old where specific commissioning conditions within a National Institute for Health and Care Excellence (NICE) Technology Appraisal, an All Wales Medicines Strategy Group (AWMSG) recommendation, or NHS Wales Joint Commissioning Committee (NWJCC) clinical policy are met, in accordance with the criteria outlined in this document.

NWJCC has carefully reviewed the evidence to treat aged under 18 years with medicines available for adults by a NICE TA/HST or AWMSG recommendation or NWJCC clinical policy. We have concluded that there is enough evidence to make these treatments routinely available to patients aged less than 18 years in certain situations.

Disclaimer

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

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.

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

Welsh Language

NWJCC is committed to treating the English and Welsh languages on the basis of equality, and endeavour to ensure commissioned services meet the requirements of the legislative framework for Welsh Language, including the Welsh Language Act (1993), the Welsh Language (Wales) Measure 2011 and the Welsh Language Standards (No.7) Regulations 2018.

Where a service is provided in a private facility or in a hospital outside of Wales, the provisions of the Welsh language standards do not directly apply but in recognition of its importance to the patient experience, the referring health board should ensure that wherever possible patients have access to their preferred language.

In order to facilitate this, NWJCC is committed to working closely with providers to ensure that in the absence of a Welsh speaker, written information will be offered and people

have access to either a translator or 'Language-line' if requested. Where possible, links to local teams should be maintained during the period of care.

Decarbonisation

NWJCC is committed to taking assertive action to reducing the carbon footprint through mindful commissioning activities. Where possible and taking into account each individual patient's needs, services are provided closer to home, including via digital and virtual access, with a delivery chain for service provision and associated capital that reflects the NWJCC commitment

1. Introduction

1.1 Plain Language Summary

Paediatric patients in specialised services should have access to medicines that have been appropriately evaluated for their use. However, safe and effective pharmacotherapy in paediatric patients requires the timely development of information on the proposed use of medicinal products in various age ranges and the development of paediatric formulations of those products.

The paediatric population should ideally be included when a product is being developed for a disease or condition in adults, especially where this is available clinical evidence. In the case of NHS Wales Joint Commissioning Committee Policies (clinical policies), the reasons for not including any ages will be detailed within the clinical policy and the Equality and Health Impact Assessment completed for each clinical policy. An EU paediatric regulation was published in 2007 by the European Medicines Agency (EMA), which sought to drive licensing of medicines for children through an incentive/reward system of patent extension. Companies seeking a licence for their product in the EU/UK are obliged to develop a Paediatric Investigation Plan (PIP) or obtain a waiver excluding them from developing a PIP. However, a paediatric licence is often sought after the adult indication has received a Marketing Authorisation (MA) and in many cases is never obtained.

The NHS Wales Individual Patient Funding Request (IPFR) Team occasionally receives requests for treatment for paediatric patients where the treatment requested is either approved by NICE or NHS Wales in the adult population. An example of such requests include

- Request for a treatment for ulcerative colitis where the medicine in question is approved by NICE in adults.

In line with the IPFR Standard Operating Procedure these requests are screened and in general will be considered as part of a cohort request and are sometimes taken forward by the ONE Wales Medicines Assessment Group (OWMAG).

NICE review medicines in line with their MA and therefore if the medicine only has a license for use in adults, NICE is unable to make recommendations for the paediatric population. This is also the case with an NWJCC clinical policy unless it is specific to the paediatric population or specifies that it covers all ages.

This policy addresses NHS Wales's position on commissioning medicines for children within specialised services where a medicine is approved for use by a NICE TA or a AWMSG recommendation or through an NWJCC clinical policy for the treatment of adults but not children.

1.2 Commissioning medicines for children in specialised services

Recommendations made by The National Institute for Health and Clinical Excellence (NICE) within their Technology Appraisals (TA)/Highly Specialised Technology Appraisals (HST) and the AWMSG recommendations only provide guidance on using a medicine in the group of patient for which the medicine has been granted a license (this may also be the case with NWJCC clinical policy). Medicines often only have a license for patients who are 18 years and above because these are the group of patients on whom the medicine has been researched. Although a patient aged under 18 years may be in a situation outlined by the TA/HST, AWMSG guideline or NWJCC clinical policy, they may not be able to access the medicine because the guidance/policy does not cover people of their age.

1.3 Epidemiology and needs assessment

There are approximately 700,000 children and young people below the age of 18 years in Wales (source Wales Census 2021). It is likely that approximately 35,000 (5%) are post pubescent. Every year, about 10% of hospital admissions involve children below the age of 18 years.

A range of medicines used to treat children are not licensed for any indication, for either adults or children (as an imported medicine, an extemporaneously prepared medicine, a medicine prepared under a special manufacturing licence, or a manipulated medicine) or are prescribed (off label) outside the terms of the product licence applying to the indication, age, dose or route of administration. Unlicensed and off label use of medicines in children range from 11% in the community to about 90% in specialist areas such as Neonatal Critical Care and on average 50% of children admitted to hospital receive either an unlicensed or off label medicine during the admission process with the most common reason for off label prescribing linked to the age of the patient.

1.4 Current Treatments

At present, patients aged under 18 years may not be able to access a medicine because a NICE TA/HST, AWMSG guidance or NWJCC clinical policy, only covers patients over 18 years of age and the only way they are able to receive these treatments is by applying to the NHS Wales Joint Commissioning Committee Individual Patient Funding Request (IPFR) process. This process however is limited in that a clinician has to demonstrate the patient presents with exceptional clinical circumstances to be considered for funding of the treatment.

1.5 What NHS Wales has decided

NWJCC has carefully reviewed the evidence to treat patients aged under 18 years with medicines available for adults by a NICE TA/HST, AWMSG recommendations or NWJCC

Clinical Policy. We have concluded that there is enough evidence to consider making these treatments routinely available to patients aged less than 18 years in certain situations.

This policy outlines that patients aged less than 18 years who meet the conditions set out in a NICE TA/HST, AWMSG recommendations or NWJCC Clinical Policy relating to adults will be able to receive the medicines without going through the IPFR process, if they meet the criteria and conditions outlined within this document.

This policy does not apply to Advanced Therapies Medicinal Products (ATMPs). Please refer to the individual ATMP Policy Position Statement for NWJCC funded eligibility criteria. If the patient does not meet the criteria as outlined in a NWJCC policy, please see section 2.6 below.

2. Criteria for Commissioning

2.1 Implementation

NHS Wales JCC will fund requests for medicines for children within a specialised service that are approved in adults by a NICE TA or AWMSG recommendation or a NWJCC clinical policy when one or more of the three following criteria are met and all of the conditions listed apply:

1. The medicine has a licence for use in children and both the indication for use and the age of the child fall within those specified in the adult licence

or

2. The medicine is listed in the BNF for Children with a recommended dosage schedule **relative to the age of the child**

or

3. The child is post pubescent.

In addition to the above criteria, **ALL** of the following conditions must apply:

- The patient meets all the NICE TA/AWMSG recommendations or NWJCC clinical policy criteria for the proposed medicine/indication.
- The patient does not meet any exclusion criteria for the medicine/indication in question.
- The use of the drug has been discussed at a specialised multidisciplinary team (MDT) meeting (including services commissioned as part of a combined adult and children's specialised service such as specialised Dermatology). At least two consultants must be involved from the relevant subspecialty with active and credible expertise in the relevant field, including at least one consultant paediatrician or a consultant with a Certificate of Completion of Training (CCT) which includes training in caring for children. In some specialities, it may be the case that medical consultants are trained in both adult and paediatric medicine. The MDT should include a paediatric pharmacist and other professional groups appropriate to the disease area.
- The patient has been registered via the NHS Wales prior approval web-based system.

In all cases the use of the medicine when off label must go through internal Trust approval systems to ensure the request is clinically safe and approved by the Trust's governance process, e.g. by its Drugs and Therapeutics Committee. It should be noted that where a medicine has an MA for use in children it should be considered prior to a funding request for a product that is not licensed for use in children.

2.2 Patient Pathway (Annex i)

It is proposed that decisions about commencing, monitoring, and stopping a treatment approved under this policy will be made by the relevant commissioned specialised

children's service or relevant specialised adult and children's service (e.g. specialised dermatology service) and in conjunction with the adult service if appropriate. The decision to prescribe the medicine must be made by an appropriately constituted specialised MDT. NHS Wales reserves the right to request evidence that processes are in place to ensure that appropriate constituted MDTs are in place.

Patients who do not meet the criteria and conditions set out in this policy can have their case considered through the NHS Wales Joint Commissioning Committee IPFR process.

2.3 Governance arrangements

Each provider organisation treating children with a medicine approved under this policy will be required to assure itself that its internal governance arrangements have been completed before the medicine is prescribed. NHS Wales JCC can ask for documented evidence that these processes are in place.

Provider organisations must seek prior approval for all patients using software such as Blueteq™ and ensure monitoring arrangements are in place to demonstrate compliance against the criteria and conditions as outlined.

2.4 Mechanism for funding

NWJCC will be responsible for commissioning treatments prescribed in line with this policy on behalf of the population of Wales.

2.4.1. Blueteq and reimbursement

Treatments prescribed for children within NWJCC commissioned specialised services, will only be funded for patients registered via the Blueteq system. Where the patient meets the criteria listed within section 2.1, a Blueteq form should be completed for approval.

If the drug treatment is stopped, it is the responsibility of the prescribing team to discontinue the Blueteq form.

For further information on accessing and completing the Blueteq form please contact NWJCC using the following e-mail address: NWJCCblueteq@wales.nhs.uk

If a non-contracted provider wishes to treat a patient that meets the criteria listed within this commissioning policy, they should contact NWJCC (e-mail: nwjccipc@wales.nhs.uk). They will be asked to demonstrate they have an appropriate MDT in place.

Funding is approved on the basis that the lowest acquisition cost drug is prescribed. It is expected that generic medicines and best value biological medicines are prescribed where available and appropriate.

2.5 Clinical Outcome and Quality Measures

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

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

All use of a biologic medicine must be entered onto the appropriate biologic registry.

2.6 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, an IPFR should be submitted.

Further information on making IPFR requests can be found at: Individual Patient Funding Requests

2.7 Responsibilities

Referrers should:

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

Clinicians considering treatment should:

- discuss all alternative treatments with the patient and/or their parent or guardian;
- advise the patient and/or their parent or guardian of any side effects and risks of the potential treatment
- inform the patient and/or their parent or guardian that treatment is not routinely funded outside of the criteria in the policy, and
- confirm that there is contractual agreement with NWJCC for the treatment and complete a Blueteq™ form where applicable.
- Complete the relevant off label safety & governance paperwork as per health board local policy.

3. Evidence

3.1 Relationship with other documents

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

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

- **All Wales Medicines Strategy Group**
  - Appraisal of paediatric minor licence extensions
  - AWMSG launches new process for appraising medicines licensed for use in children
  - Maximising the opportunity presented by biosimilar medicines – A national strategy for Wales

- **Relevant NHS England policies**
  - Commissioning Medicines for Children in Specialised Services, March 2024

3.2 References

1. Pediatric Decision Tree. US Food and Drug Administration. Specific requirements on content and format of labelling for human prescription drugs: revision of "pediatric use" subsection in the labelling: final rule. Fed Regist. 1994;59.
2. Manolis E, Pons G (2009) Proposals for model based paediatric medicinal development within the current EU regulatory framework. Br J Clin Pharmacol 68: 493-501.
3. Extrapolation of adult data and other data in pediatric drug-development programs. Dunne J, Rodriguez WJ, Murphy MD, Beasley BN, Burckart GJ, Filie JD, Lewis LL, Sachs HC, Sheridan PH, Starke P, Yao LP. Pediatrics. 2011 Nov;128(5):e1242-9.
4. BNF for Children
5. Conroy et al (2000) Survey of unlicensed and off label use of drugs in paediatric wards in European countries Br Med J 320:79.
6. Pandolfini et al (2005) A literature review on off-label drug use in children Eur J Paed 164(9): 552-8.
7. Royal Pharmaceutical Society: A competency based framework for all Prescribers. Published September 2021 [accessed on 5 August 2024]
8. National prescribing indicators 2022-2025: Supporting information for prescribers and healthcare professionals. DRAFT [access on 5 August 2024]

3.3 Date of Review

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

If an update is carried out the policy will remain extant until the revised policy is published.

4. 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 NHS Wales Joint Commissioning 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.

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 NWJCC.

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.

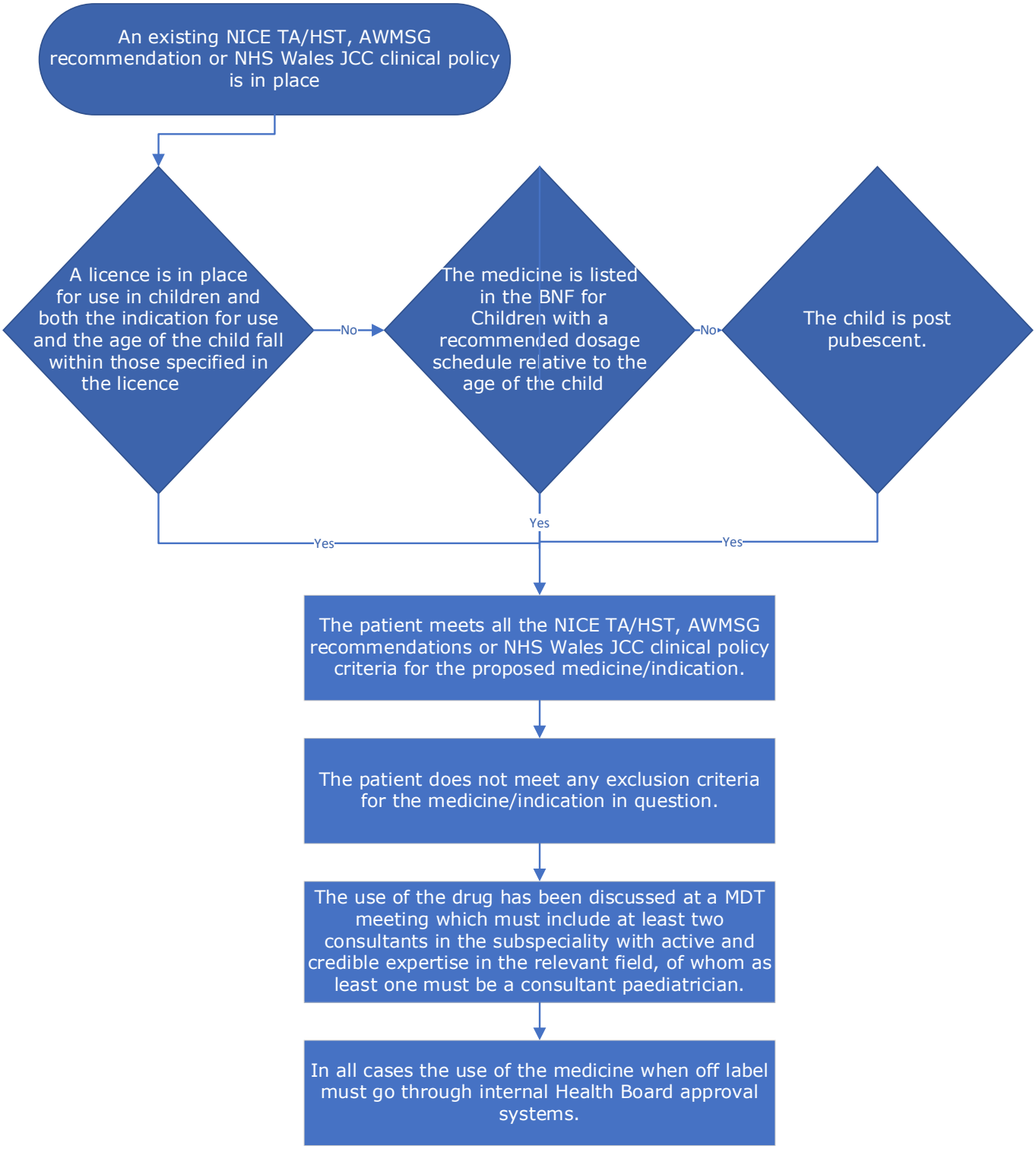
If an IPFR is declined by the Panel, a patient and/or their NHS clinician has the right to request information about how the decision was reached. If the patient and their NHS clinician feel the process has not been followed in accordance with this policy, arrangements can be made for an independent 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, and IPFR should be submitted.

Further information on making IPFR requests can be found at: Individual Patient Funding Requests

Annex i Patient Pathway

Patient Pathway Treatment algorithm for medicines being considered under this policy



Annex ii Abbreviations and Glossary

Abbreviations

AWMSG All Wales Medicines Strategy Group

IPFR Individual Patient Funding Request

NWJCC NHS Wales Joint Commissioning Committee

BNF (for Children) British National Formulary (for Children)

Glossary

Individual Patient Funding Request (IPFR)

An IPFR is a request to NHS Wales Joint Commissioning Committee (NWJCC) to fund an intervention, device or treatment for patients that fall outside the range of services and treatments routinely provided across Wales.

NHS Wales Joint Commissioning Committee (NWJCC)

NWJCC is a joint committee of the seven local health boards in Wales. The purpose of NWJCC is to ensure that the population of Wales has fair and equitable access to the full range of Tertiary Services. NWJCC ensures that services within our portfolio 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.

British National Formulary (BNF) for Children

The BNF for Children is for reference by UK health professionals engaged in prescribing, dispensing, and administering medicines to children.

Off label

A term used to describe the use of a licensed medicine outside the terms of its marketing authorisation e.g. on the basis of age, dose, route, indication.

Specialised service

A service that is defined within the Specialised Service - service Specification.