GIG CYMRU NHS WALES
Cyd-bwyllgor Comisiynu
Joint Commissioning Committee

Drug treatments for seizures associated with Dravet syndrome or Lennox-Gastaut syndrome in people aged 2 years and older

Policy Position Statement: PPS203

Document Information	
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Target Audience	Chief Executives, Medical Directors, Directors of Finance, Consultant Neurosurgeons, Director of Operations for Specialist Services, Directorate Manager for Neurosciences and Paediatrics, Consultant Paediatricians, Community Paediatricians, Director of Nursing, Lead Nurses, Specialist Epilepsy Nurses, Consultant Neurologists, Lead Pharmacists, Head of Finance and Commissioning, Health Board Commissioning Managers, Planning Managers, Directors of Pharmacy, Drug manufacturers
Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy
Document Update Information	March 2025: Scope of commissioning for fenfluramine extended to include 'Fenfluramine for treating seizures associated with Lennox–Gastaut syndrome in people 2 years and over (TA1050)'

Contents

Policy Statement

NHS Wales Joint Commissioning Committee (NWJCC) approve the funding of the following drug treatments for people aged 2 years to 16 years in accordance with the criteria outlined in this document:

- cannabidiol with clobazam for treating seizures associated with Dravet syndrome,
- cannabidiol with clobazam for treating seizures associated with Lennox–Gastaut syndrome
- fenfluramine for treating seizures associated with Dravet syndrome
- fenfluramine for treating seizures associated with Lennox-Gastaut syndrome.

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.

Disclaimer

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

This document 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.

1. Introduction

This Policy Position Statement has been developed for the planning and delivery of the following drug treatments for people aged 2 years to 16 years resident in Wales:

- cannabidiol with clobazam for treating seizures associated with Dravet syndrome
- cannabidiol with clobazam for treating seizures associated with Lennox–Gastaut syndrome
- fenfluramine for treating seizures associated with Dravet syndrome
- fenfluramine for treating seizures associated with Lennox-Gastaut syndrome.

This service will only be commissioned by the NHS Wales Joint Commissioning Committee (NWJCC) and applies to residents of all seven Health Boards in Wales.

In creating this document NWJCC has reviewed the relevant guidance issued by the National Institute of Health and Care Excellence (NICE)[1,2,3] and has concluded the drugs listed above should be made available.

1.1 Background

Epilepsy is a common condition that affects the brain and causes seizures, which are bursts of electrical activity in the brain which have a range of symptoms and affects how the brain works. In epilepsy, certain signs and symptoms added together would suggest that the child has a particular medical condition known as a syndrome and this is dependent on the child's age at which seizures begin, the type of seizure, frequency and whether they are male or female and have learning difficulties.

Dravet syndrome is an epilepsy syndrome that begins in infancy or early childhood and can include a spectrum of symptoms ranging from mild to severe. Children with Dravet syndrome initially show focal (confined to one area) or generalized (throughout the brain) convulsive seizures that start before 15 months of age (often before age one). Dravet syndrome is a rare, lifelong form of epilepsy[4]. It affects around 1 in 15,000 children in the UK[5]. It can cause developmental delays and learning difficulties.

Lennox-Gastaut syndrome is a severe form of epilepsy. Seizures begin in early childhood, usually before the age of 4 years. Children, adolescents, and adults with Lennox-Gastaut

[1] Overview | Cannabidiol with clobazam for treating seizures associated with Dravet syndrome | Guidance | NICE
[2] Overview | Cannabidiol with clobazam for treating seizures associated with Lennox–Gastaut syndrome | Guidance | NICE
[3] Overview | Fenfluramine for treating seizures associated with Dravet syndrome | Guidance | NICE
[4] Dravet Syndrome | National Institute of Neurological Disorders and Stroke (nih.gov)
[5] Dravet syndrome - Epilepsy Action

syndrome have multiple types of seizures that vary among individuals. Common seizure types include:

- tonic seizures (stiffening of the body, upward eye gaze, dilated pupils, and altered breathing patterns)
- atypical absences (staring spells)
- atonic seizures (brief loss of muscle tone, which could cause abrupt falls)
- myoclonic seizures (sudden muscle jerks)
- generalized tonic-clonic seizures (muscle stiffness and rhythmic jerking)

There may be periods of frequent seizures mixed with relatively seizure-free periods. Although not always present at the onset of seizures, most people living with Lennox-Gastaut syndrome experience some degree of impaired intellectual functioning or information processing, along with developmental delays and behavioural disturbances[6]. Lennox-Gastaut syndrome affects around 1 or 2 in every 100 children with epilepsy[7].

The most common treatment used to treat epilepsy in UK clinical practice is the use of anti-epileptic drugs, (AEDs) to suppress seizures. According to NICE clinical guideline for Epilepsies, diagnosis and management (CG137)[8], the AED treatment strategy should be individualised according to the epilepsy syndrome, seizure type, co-medication, co-morbidity, the person's lifestyle, and the preferences of the person and their family and/or carers. Children with either Dravet syndrome or Lennox-Gastaut syndrome should have specialist input into their management.

Current treatment options for treating children with seizures associated with and Dravet syndrome and Lennox-Gastaut syndrome includes the use of AEDs, however if seizures cannot be controlled well enough after trying two or more AEDs, patients can be treated with either cannabidiol with clobazam or fenfluramine for Dravet syndrome and cannabidiol with clobazam or fenfluramine for Lennox-Gastaut syndrome accordingly. Non-pharmacological treatment options include a ketogenic diet, vagus nerve stimulation and various other surgical procedures.

1.2 Equality Impact Assessment

The Equality Impact Assessment (EIA) 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).

[6] Lennox-Gastaut Syndrome | National Institute of Neurological Disorders and Stroke (nih.gov)
[7] Lennox-Gastaut syndrome - Epilepsy Action
[8] Overview | Epilepsies: diagnosis and management | Guidance | NICE

An EQIA was carried out by NICE during the evaluation of:

- cannabidiol with clobazam for treating seizures associated with Dravet syndrome NICE EQIA cannabidiol for treating Dravet Syndrome
- cannabidiol with clobazam for treating seizures associated with Lennox–Gastaut syndrome NICE EQIA cannabidiol for treating Lennox Gastaut
- fenfluramine for treating seizures associated with Dravet syndrome NICE EQIA Fenfluramine for treating Dravet Syndrome
- fenfluramine for treating seizures associated with Lennox-Gastaut syndrome NICE EQIA fenfluramine for treating Lennox Gastaut

2. Recommendations

The recommendations below represent the views of NICE, arrived at after careful consideration of the evidence available. Health professionals are expected to take into account the relevant NICE guidance, alongside the individual needs, preferences and values of the patient.

2.1 Inclusion Criteria

Cannabidiol with clobazam for treating seizures associated with Dravet syndrome (TA614)

Cannabidiol with clobazam is recommended as an option for treating seizures associated with Dravet syndrome in people aged 2 years and older, only if:

- the frequency of convulsive seizures is checked every 6 months, and cannabidiol is stopped if the frequency has not fallen by at least 30% compared with the 6 months before starting treatment
- the company provides cannabidiol according to the commercial arrangement[9].

Cannabidiol with clobazam for treating seizures associated with Lennox–Gastaut syndrome (TA615)

Cannabidiol with clobazam is recommended as an option for treating seizures associated with Lennox-Gastaut syndrome in people aged 2 years and older, only if:

- the frequency of drop seizures is checked every 6 months, and cannabidiol is stopped if the frequency has not fallen by at least 30% compared with the 6 months before starting treatment
- the company provides cannabidiol according to the commercial arrangement[10].

Fenfluramine for treating seizures associated with Dravet syndrome (TA808)

Fenfluramine is recommended as an add-on to other antiseizure medicines for treating seizures associated with Dravet syndrome in people aged 2 years and older, only if:

- seizures have not been controlled after trying 2 or more antiseizure medicines
- the frequency of convulsive seizures is checked every 6 months, and fenfluramine is stopped if the frequency has not fallen by at least 30% compared with the 6 months before starting treatment
- the company provides fenfluramine according to the commercial arrangement[11].

[9] Overview | Cannabidiol with clobazam for treating seizures associated with Dravet syndrome | Guidance | NICE
[10] Overview | Cannabidiol with clobazam for treating seizures associated with Lennox–Gastaut syndrome | Guidance | NICE
[11] Overview | Fenfluramine for treating seizures associated with Dravet syndrome | Guidance | NICE

Fenfluramine for treating seizures associated with Lennox-Gastaut syndrome (TA1050)

Fenfluramine is recommended as an option for treating seizures associated with Lennox–Gastaut syndrome (LGS), as an add-on to other antiseizure medicines, for people 2 years and over. It is recommended only if:

- the frequency of drop seizures is checked every 6 months, and fenfluramine is stopped if the frequency is not reduced by at least 30% compared with the 6 months before starting treatment
- the company provides it according to the commercial arrangement.

This recommendation is not intended to affect treatment with fenfluramine that was started in the NHS before this guidance was published. People having treatment outside this recommendation may continue without change to the funding arrangements in place for them before this guidance was published, until they and their NHS healthcare professional consider it appropriate to stop. For children or young people, this decision should be made jointly by the healthcare professional, the child or young person, and their parents or carers[12].

This Policy Position Statement applies to children aged 2-16 years of age.

2.2 Exclusion Criteria

Patients under 2 years of age.

2.3 Stopping Criteria

The frequency of convulsive seizures should be checked every 6 months, and cannabidiol / fenfluramine should be stopped if the frequency has not fallen by at least 30% compared with the 6 months before starting treatment.

2.4 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.

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.

[12] Overview | Fenfluramine for treating seizures associated with Lennox–Gastaut syndrome in people 2 years and over | Guidance | NICE

2.5 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.

2.6 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.

2.7 Designated Providers

South Wales
Noah's Ark Children's Hospital for Wales
Cardiff and Vale University Health Board
Heath Park Way
Cardiff
CF14 4XW

Powys
Birmingham Children's Hospital
Steelhouse Lane
Birmingham
B4 6NH

North Wales
Alder Hey Children's Hospital
East Prescot Road

Liverpool
L14 5AB

2.8 Patient Pathway (Annex i)

The diagnosis of epilepsy in children aged 2 years and older is undertaken by an experienced Medical Practitioner with training and expertise in epilepsy.

A number of investigations and assessments are undertaken to understand the seizure type and epilepsy syndrome. It may be necessary for patients to have an electroencephalogram (EEG). Long-term video or ambulatory EEG may be used in the assessment of children who present diagnostic difficulties after clinical assessment of a standard EEG.[13] Other investigations include Neuroimaging, Neuropsychological assessment and biochemistry tests to support with the diagnosis of the condition.

Patients should be referred to a Paediatric Neurologists at the designated provider centre listed within this policy (section 2.7), for assessment of eligibility for treatment(s) included in this policy.

Children aged 2 years and older should receive this treatment in a child-centred environment.

2.9 Mechanism for funding

The drug treatments listed within this policy as being commissioned, will only be funded for patients registered via the Blueteq® system and where an appropriately constructed MDT has approved its use within highly specialised centres. Where the patient meets 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 NWJCC using the following email address: NWJCCblueteq@wales.nhs.uk.

If a non-contracted provider wishes to treat a patient that meets the criteria they should contact NWJCC at 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 drug treatments listed within this policy are prescribed and administered in accordance with its marketing authorisation.

Cannabidiol, is available as 100mg/ml oral solution. The cost is £850.29 for a 100ml bottle (excluding VAT; company's evidence submission). The company has a commercial

[13] 1 Guidance | Epilepsies: diagnosis and management | Guidance | NICE

arrangement. This makes the drug treatments listed within this policy 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 Commercial Medicines Access References Tool (CMART) for further information on the Patient Access Scheme (PAS) price.

Fenfluramine, is available as fenfluramine 2.2mg/ml liquid. The cost is £1802.88 for a 120 ml bottle and £5,408.65 for a 360ml bottle (excluding VAT; company's evidence submission). The company has a commercial arrangement. This makes the drug treatments listed within this policy 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 Commercial Medicines Access References Tool (CMART) for further information on the Patient Access Scheme (PAS) price.

If treatment is discontinued, it is the responsibility of the prescribing team to discontinue the Blueteq® form.

Where available and suitable, a homecare provider should be used for medication supply. In the event of a homecare provider not being a suitable option for the patient, a local centre under the instruction of the specialised centre will be reimbursed for ongoing medicines supply providing that a continuation Blueteq® form is completed.

2.10 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.

2.11 Action to be taken

- Health Boards and NWJCC are to circulate this Policy Position Statement to all Hospitals/MDTs to inform them of the conditions under which the treatment will be commissioned.
- Providers are to ensure that all providers are purchasing the drug treatments included in this policy at the agreed discounted price.
- Providers are to ensure the need to approve the drug treatments included in this policy 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.
- The Provider should work to written quality standards and provide monitoring information to NWJCC on request.

3. Putting things right

3.1 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.

3.2 Individual Patient Funding Request (IPFR)

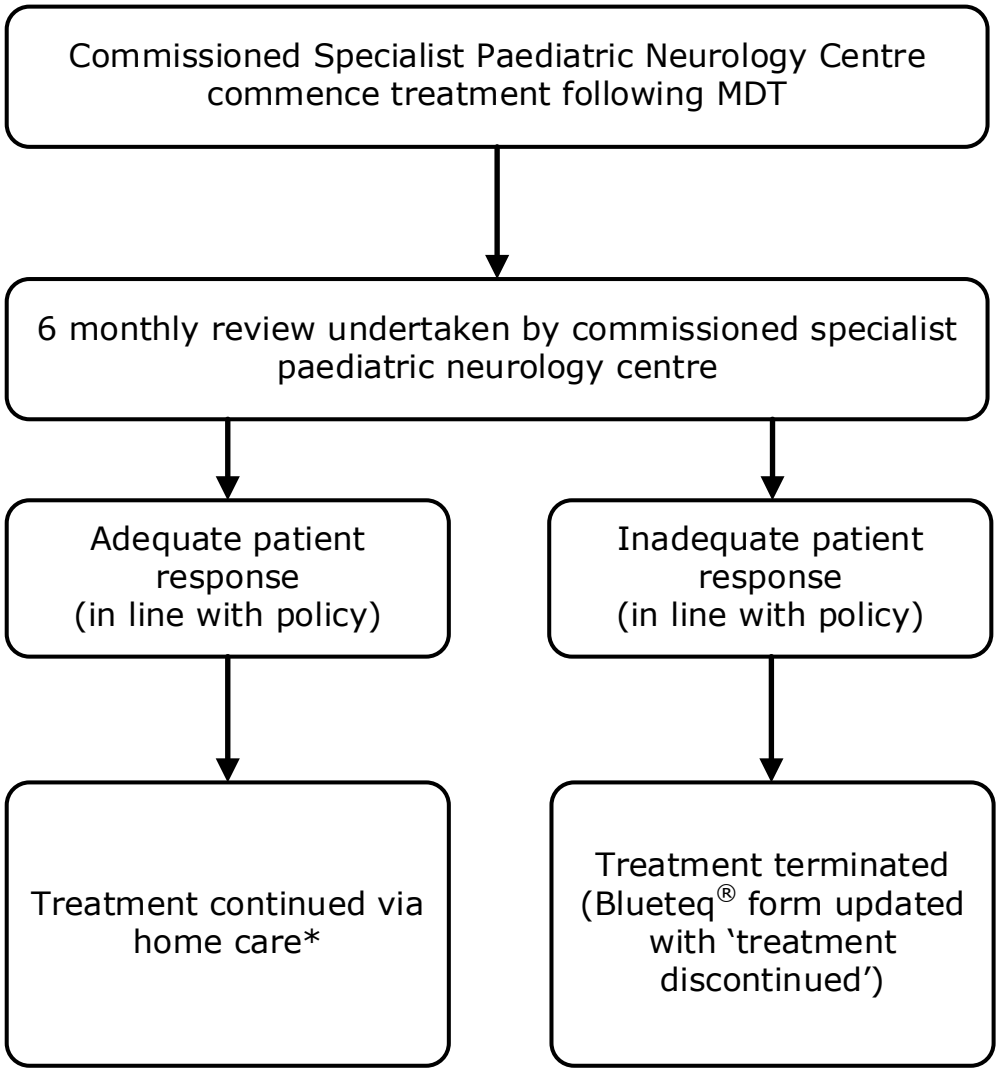
If the patient does not meet the criteria for treatment as outlined in this policy, an Individual Patient Funding Request (IPFR) can be submitted for consideration in line with the All Wales Policy: Making Decisions on Individual Patient Funding Requests. The request will then be considered by the All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



*Where available and suitable, a homecare provider should be used for medication supply

Annex ii Codes

The list of ICD codes is indicative and is not exhaustive. Additional codes may be used for contract monitoring purposes, furthermore some codes may cover indications not included within this policy.

Code Category	Code	Description
ICD	G40	Epilepsy and recurrent seizures

Contact Us

If you have a question related to this document you can contact us using one of the methods outlined below.

If you would like this document in an alternative format and/or language, please contact us for assistance.

Email:

NWJCC consultation mailbox – nwjccconsultation@wales.nhs.uk

Telephone:

General Enquiries – 01443 433112

Website:

Contact us - NHS Wales Joint Commissioning Committee

Writing:

If you wish to contact the NHS Wales Joint Commissioning Committee, you can write to us at one of our locations below, we welcome correspondence in Welsh or English:

South Wales Offices
Unit 1, Charnwood Court, Heol Billingsley, Nantgarw, CF15 7QZ
Unit G1 The Willowford, Main Avenue, Treforest Industrial Estate, Pontypridd, CF37 5YL

North Wales Offices
Unit 3, Media Point - Unit 3, Mold Business Park, Mold, CH7 1XY
Preswylfa, Hendy Road, Mold, CH7 1PZ