GIG CYMRU NHS WALES
Cyd-bwyllgor Comisiynu
Joint Commissioning Committee

Evinacumab for the treatment of homozygous familial hypercholesterolaemia in people aged 12 years and over

Policy Position Statement: PPS308

Document Information	
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Target Audience	Chief Executives, Medical Directors, Directors of Finance, Ultragenyx UK, Paediatric clinical leads, Lipid clinical leads, Directors of pharmacy, Lead Paediatric pharmacists, Pharmacists for inherited metabolic disorders, Cardiologists
Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy

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Policy Statement

The NHS Wales Joint Commissioning Committee (NWJCC) will commission evinacumab for people with homozygous familial hypercholesterolaemia in people aged 12 years and over in accordance with the criteria outlined in this document.

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 evinacumab for homozygous familial hypercholesterolaemia for people aged 12 years and over resident in Wales. 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] and has concluded that evinacumab should be made available.

1.1 Background

Familial hypercholesterolaemia (FH) is an inherited genetic condition passed down through families[2]. It can be divided into heterozygous FH (more common) and homozygous FH (less common but more severe). This policy only covers treatment of homozygous FH. Homozygous FH occurs due to inheriting mutations in genes from both parents[3]. These mutations reduce the liver's ability to remove 'bad' cholesterol, known as low density lipoprotein (LDL)[2]. There are a number of different genes involved in homozygous HF[4].

Normally the cells in the liver (and certain other cells in your body) that remove LDL cholesterol have extensions on them called LDL receptors. Their job is to catch the LDL cholesterol as it passes by in the blood and take it into the cell to be used, stored or broken down[4]. However, individuals with homozygous FH will either have faulty LDL receptors or a decreased number of LDL receptors. This means that the LDL cholesterol is not cleared from the blood resulting in high levels in the blood[4]. This increases the risk of heart and circulatory disease at an early age, even in childhood[2,4].

FH remains underdiagnosed and undertreated[5]. People with homozygous FH should start treatment as soon as possible as early identification and treatment can improve prognosis considerably. FH cannot be treated by diet and exercise alone. These lifestyle changes can help, but medications are recommended to reduce LDL cholesterol levels by at least 50%[5].

[1] Overview | Evinacumab for treating homozygous familial hypercholesterolaemia in people 12 years and over | Guidance | NICE
[2] Familial hypercholesterolaemia: symptoms, causes and treatments - BHF
[3] Homozygous Familial Hypercholesterolemia: Diagnosis and Emerging Therapies - American College of Cardiology (acc.org)
[4] What is HoFH? What is Familial Hypercholesterolaemia? HEART UK
[5] What is Familial Hypercholesterolemia? | American Heart Association

Standard treatment usually involves statins and other cholesterol-lowering medications; ezetimibe, bile acid sequestrants (cholestyramine or colesevelam) and PCSK9 inhibitors. People with extremely high LDL cholesterol, such as those with homozygous FH, may need a treatment called LDL apheresis. This is a dialysis-like procedure that's done every few weeks to remove cholesterol from the blood by filtering it out.

Evinacumab is a recombinant human monoclonal antibody[6]. Monoclonal antibodies are proteins made in laboratories that mimic natural proteins in our bodies called antibodies[7]. Evinacumab works by binding to and blocking the angiopoeitin-like 3 protein (ANGPTL3) to prevent inhibition of lipoprotein lipase and endothelial lipase. This leads to increased removal of cholesterol-containing lipoproteins from the blood.

Homozygous FH is considered an extremely rare disease with an estimated prevalence of around 1 per 1 million adults in the United Kingdom (UK)[8], equating to around 3 adults in Wales. However, more recent studies suggest a higher prevalence of around 1:300,000[9].The prevalence of young people with homozygous FH is likely to be minimal with an estimated number of between 10–12 in the UK[1,] however this will be dependent on when the person is diagnosed. It is estimated there will be around 1 new case per year in England[10].

1.2 Equality Impact 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).

An EQIA was carried out by NICE during the evaluation of evinacumab for the treatment of homozygous familial hypercholesterolaemia. For further details, please refer to the NICE website at: NICE EQIA

[1] Overview | Evinacumab for treating homozygous familial hypercholesterolaemia in people 12 years and over | Guidance | NICE
[6] Evinacumab | Drugs | BNF | NICE
[7] Monoclonal Antibodies: Definition & How Treatment Works (clevelandclinic.org)
[8] 1679-lomitapide.pdf
[9] Homozygous autosomal dominant hypercholesterolaemia in the Netherlands: prevalence, genotype-phenotype relationship, and clinical outcome - PubMed
[10] https://www.nice.org.uk/guidance/ta1002/documents/1

2. Recommendations

The NHS Wales Joint Commissioning Committee approve funding of evinacumab for the treatment of homozygous familial hypercholesterolemia in people aged 12 years and over in line with the criteria outlined in this policy.

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[1], alongside the individual needs, preferences and values of the patient.

2.1 Inclusion Criteria

Evinacumab alongside diet and other low-density lipoprotein-cholesterol (LDL-C) lowering therapies is recommended, within its marketing authorisation, as an option for treating homozygous FH in people 12 years and over. It is only recommended if the company provides it according to the commercial arrangement[1].

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.

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 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.

2.5 Designated Providers

Patients will be referred to one of the designated centres below for treatment:

Paediatric Providers

Childrens Hospital for Wales/
University Hospital of Wales
Heath Park
Cardiff
CF14 4XW

Birmingham Womens and Childrens Hospital
Mindelsohn Way
Birmingham
B15 2TG

Royal Manchester Children's Hospital
Oxford Road
Manchester
M13 9WL

Patients seen at the Royal Manchester Children's Hospital may be followed up at outreach clinics at Alder Hey Children's Hospital.

Adult Providers

University Hospital of Wales
Heath Park,
Cardiff,
CF14 4XW
(The adult service will be located at the Llandough site)

Manchester Royal Infirmary
Oxford Road

Manchester
M13 9WL

2.6 Patient Pathway (Annex i)

Patients should be referred to one of the designated providers listed in section 2.5 for assessment of eligibility for treatment included in this policy. See Annex i for the patient pathway.

2.7 Mechanism for funding

Evinacumab 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 NWJCCblueteq@wales.nhs.uk. They will be asked to demonstrate they have an appropriate MDT in place.

Funding is approved on the basis that evinacumab is prescribed and administered in accordance with its marketing authorisation. Evinacumab is available as Evkeeza® 150 mg/ml concentrate for solution for infusion[11]. The cost of each 345mg/2.3ml concentrate for solution for infusion vial is £6,432.75 (excluding VAT; company's evidence submission)[12]. The company has a commercial arrangement. This makes the drug 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 Reference~ 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.

[11] Evkeeza 150 mg/ml concentrate for solution for infusion - Summary of Product Characteristics (SmPC) - (emc) (medicines.org.uk)
[12] Medicinal forms | Evinacumab | Drugs | BNF | NICE

2.8 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.

2.9 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 technology will be commissioned.
- NWJCC are to ensure that all providers are purchasing evinacumab at the agreed discounted price.
- Providers are to ensure the need to approve evinacumab 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.

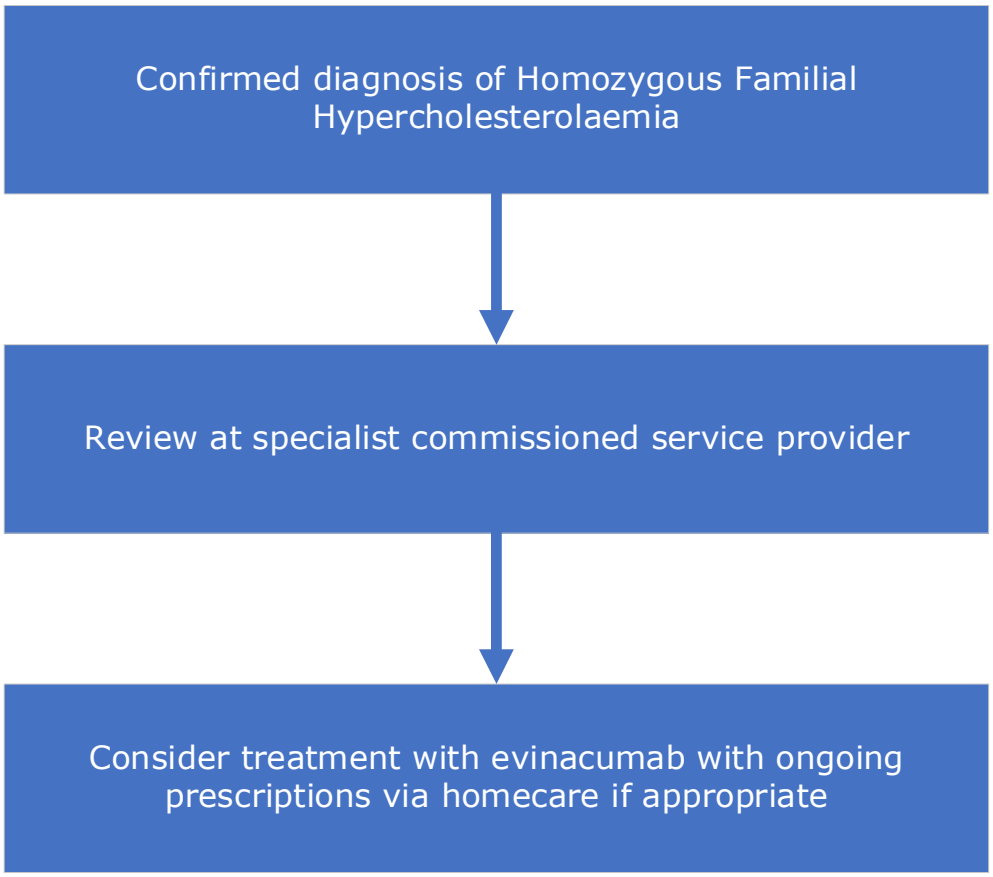
If a patient or their representative is unhappy with the care provided during the treatment or the clinical decision to withdraw treatment provided under this policy, the patient and/or their representative should be guided to the LHB for NHS Putting Things Right. For services provided outside NHS Wales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

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

Annex i Patient Pathway



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