GIG CYMRU NHS WALES
Cyd-bwyllgor Comisiynu
Joint Commissioning Committee

Emicizumab as prophylaxis in people with congenital haemophilia A with Factor VIII inhibitors (all ages)

Policy Position Statement: PPS167

Document Information	
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Target Audience	Chief Executives, Medical Directors, Directors of Finance, Consultant Haematologists, Comprehensive Care Centre Directors, Haemophilia Centre Directors
Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy
Policy Update Information	Reference to dosing regimen changed in section 1.5. Off Label Use section has been removed. Reference to homecare added under patient pathway.

Contents

Abbreviations

AWMSG	All Wales Medicines Strategy Group
IPFR	Individual Patient Funding Request
ITI	Immune Tolerance Induction
NWJCC	NHS Wales Joint Commissioning Committee
UKHDCO	United Kingdom Haemophilia Centre Doctor's Organisation

Policy Statement

NHS Wales Joint Commissioning Committee (NWJCC) will commission emicizumab as prophylaxis treatment for people with congenital haemophilia A with factor VIII inhibitors in accordance with the criteria outlined in this document.

In creating this document NWJCC has reviewed the relevant guidance issued by NHS England and has concluded that emicizumab as prophylaxis treatment for people with congenital haemophilia A with factor VIII inhibitors should be made available to patients in Wales.

This NWJCC policy position statement adopts the commissioning criteria of the NHS England commissioning policy, emicizumab as prophylaxis in people with congenital haemophilia A with factor VIII inhibitors (all ages)[1].

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] https://www.england.nhs.uk/wp-content/uploads/2018/07/1717-emicizumab.pdf

Disclaimer

NWJCC assumes that healthcare professionals will use their clinical judgement, 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 emicizumab as prophylaxis treatment for people with congenital haemophilia A with factor VIII inhibitors for people 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.

1.1 Plain Language summary

Haemophilia A is a rare condition that affects the blood's ability to clot. Haemophilia A is usually inherited and usually occurs in males. Instances in females are rare.

Normally, when a person cuts themselves, substances in the blood called clotting factors combine with blood cells called platelets, making the blood clot and stopping the bleeding. People with haemophilia A do not have enough of a clotting factor called factor VIII (eight) in their blood, or it isn't working properly. This means they cannot form strong clots and so they bleed for longer than usual.

Symptoms of haemophilia A can be mild to severe, depending on the level of clotting factor VIII. People with haemophilia A may bruise easily and bleed for longer than people who do not have haemophilia A. Bleeding can be external (for example, from cuts) or internal (for example, into the brain or into joints, including the knee and elbow). Bleeding into joints causes acute pain and over time irreversible damage to the joints (reducing the person's ability to move) and reduce the person's quality of life. Bleeding into the brain may be fatal.

People with severe haemophilia A are normally treated by replacing the missing factor VIII. Factor VIII replacement treatment prevents bleeds and allows the person to grow up with normal joints. Sometimes the body's immune system sees the replacement factor VIII as 'invading' the body. The body produces antibodies called 'inhibitors' to attack the replacement factor VIII, stopping it from working. This happens to around 1 in 3 people with severe haemophilia A who are treated with replacement factor VIII. Compared to people without inhibitors, people with inhibitors have a higher rate of bleeding complications (bleeds are harder to prevent and to treat) and are more likely to have joint damage.

1.2 Aims and Objectives

This Policy Position Statement aims to define the commissioning position of NWJCC on the use of emicizumab as prophylaxis treatment for people with congenital haemophilia A with factor VIII inhibitors.

The objectives of this policy are to:

- ensure commissioning for the use of emicizumab is evidence based
- ensure equitable access to emicizumab
- define criteria for people with congenital haemophilia A with factor VIII inhibitors to access treatment
- improve outcomes for people with congenital haemophilia A with factor VIII inhibitors

1.3 Epidemiology

The United Kingdom Haemophilia Centre Doctor's Organisation (UKHCDO) Bleeding Disorder Statistics for Wales reports that between April 2023 and March 2024 there were 348 people with mild, moderate or severe forms of haemophilia A (not including low-level carriers; factor VIII level <40 IU/dL). Of these, 15 people (4.3%) have current inhibitors, the majority of whom have severe haemophilia A (9 people; 60%). The eligible patient population for emicizumab is considered to be equivalent to the patients with current inhibitors.

1.4 Current Treatment

There is currently no cure for haemophilia A. Lifelong treatment is required. The aim of treatment for haemophilia A is to prevent bleeding episodes from occurring. In particular, the aim is to prevent joint bleeds (and therefore prevent joint damage) and other serious bleeds which can lead to disability and death. Bleeds can be prevented by injections of factor VIII given 3 to 4 times a week, however, this treatment is not possible for people with an inhibitor because the factor VIII does not work.

One of the main treatments for people with haemophilia A with factor VIII inhibitors is to try to eradicate the inhibitors, using a treatment called immune tolerance induction (ITI). About two-thirds of patients who develop an inhibitor can be expected to achieve inhibitor eradication following ITI.

People with an inhibitor who cannot be treated with factor VIII are treated with "bypassing agents", these activate the blood clotting system differently to factor VIII and are not affected by inhibitors. However, bypassing agents are not as good as factor VIII for preventing or treating bleeds. Bypassing agents are given by injection into a vein, or into central venous access devices (CVADs) which facilitates venous access. The 2 main ways of giving bypassing agents are:

- Preventative treatment (also called prophylaxis), the person has regular bypassing agent injections (every 2-3 days) to prevent or reduce the risk of bleeding. About two-thirds of people with haemophilia A with inhibitors in the UK are managed with a prophylactic bypassing agent regimen.

- On-demand treatment (also called episodic treatment), the person has bypassing agent injections only when a bleed occurs to stop the bleed. About one-third of people with haemophilia A and inhibitors in the UK are managed with an on-demand bypassing agent regimen.

1.5 Proposed Treatment

Emicizumab is a drug used to prevent bleeding or reduce the number of bleeds in people with haemophilia A who have factor VIII inhibitors. It is administered as a subcutaneous injection. Emicizumab works by binding to factor X and activated factor IX which brings those clotting factors near each other and activates the blood clotting system even if no factor VIII is present. This is different to how replacement factor VIII and bypassing agents work. Emicizumab is injected under the skin (subcutaneous injection). The dosing regimen is based on the persons weight and can be found in the summary of product characteristics[2].

1.6 What NHS Wales has decided

NWJCC has carefully reviewed the relevant guidance issued by NHS England. We have concluded that emicizumab should be made available within the criteria set out in section 2.1.

[2] Home - electronic medicines compendium (emc)

2. Criteria for Commissioning

The NHS Wales Joint Commissioning Committee approve the funding of emicizumab as prophylaxis treatment for people with congenital haemophilia A with factor VIII inhibitors in line with the criteria identified in this policy.

2.1 Inclusion Criteria

NWJCC will routinely commission emicizumab prophylaxis in adults and children with congenital haemophilia A and inhibitors to prevent bleeding episodes where the patient:

- has a factor VIII inhibitor confirmed on more than one occasion by a Nijmegen-modified Bethesda assay, that compromises the effect of prophylaxis or treatment of bleeds at standard doses of factor VIII

and meets at least one of the following criteria:

- has had ITI if indicated which has not been successful in eradicating the inhibitor (see section 9), **or**
- is an existing patient with poorly controlled bleeding episodes, **or**
- currently receives bypassing agents either prophylactically or on-demand, **or**
- is undergoing ITI and requires prophylaxis to prevent breakthrough bleeding episodes during ITI treatment.

2.2 Exclusion Criteria

NWJCC will only commission emicizumab prophylaxis for patients meeting the criteria in 2.1.

2.3 Stopping Criteria

Treatment with emicizumab should be withdrawn and ceased in the following situations:

- An increase in the number of bleeding episodes compared with previous treatment
- Less than 50% reduction in number of breakthrough bleeds after 6 months of maintenance prophylaxis with emicizumab compared with previous episodic (on-demand) 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.

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 Patient Pathway (Annex i)

Emicizumab will only be commissioned and funded via Haemophilia Comprehensive Care Centres. Emicizumab should only be prescribed by a Comprehensive Care Centre. All patients receiving emicizumab must have access 24 hours a day, 7 days a week to consultant haematologists with expertise in treating patients with inhibitors. Homecare should be utilised for supply if available and appropriate.

2.8 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

2.9 Clinical Outcome and Quality Measures

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

All patients must be registered with the UK National Haemophilia Database and details of their inhibitor reported as soon as they are confirmed. The outcome of emicizumab prophylaxis must be reported to the National Haemophilia Database annually. Patients receiving emicizumab must record all their bleeds and treatment on a secure therapy recording system.

All haemophilia comprehensive care centres will be required to participate in national audits, which will include:

- starting dose and dose changes to review compliance with protocols
- Factor VIII and bypassing agent usage
- number of bleeding episodes per year (and annualised baseline number of bleeding episodes before commencing emicizumab prophylaxis)
- adverse reactions (including thrombotic events and allergic reactions)

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.

2.10 Mechanism for funding

Emicizumab 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.

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

2.11 Responsibilities

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.

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.

Clinician considering treatment should:

- discuss all the alternative treatment 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.

In all other circumstances an IPFR must be submitted.

3. Documents which have informed this policy

The following documents have been used to inform this policy:

- **NHS England policies**
  - Clinical Commissioning Policy: Emicizumab as prophylaxis in people with congenital haemophilia A with factor VIII inhibitors (all ages). NHS England Ref:170067/P. July 2018

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

- UK national guidelines on Emicizumab (Collins et al Haemophilia 24:344-347, 2018).

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

- **NHS Wales Joint Commissioning Committee policies and service specifications**
  - PPS189 Emicizumab as prophylaxis in people with severe congenital haemophilia A without factor VIII inhibitors (all ages)
  - SS77 Bleeding Disorders (all ages) service specification

4. Date of Review

This document is scheduled for review every three years, unless information is received that the policy requires revision.

If an update is carried out, this version of the policy will remain extant until the revised policy is published.

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.

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

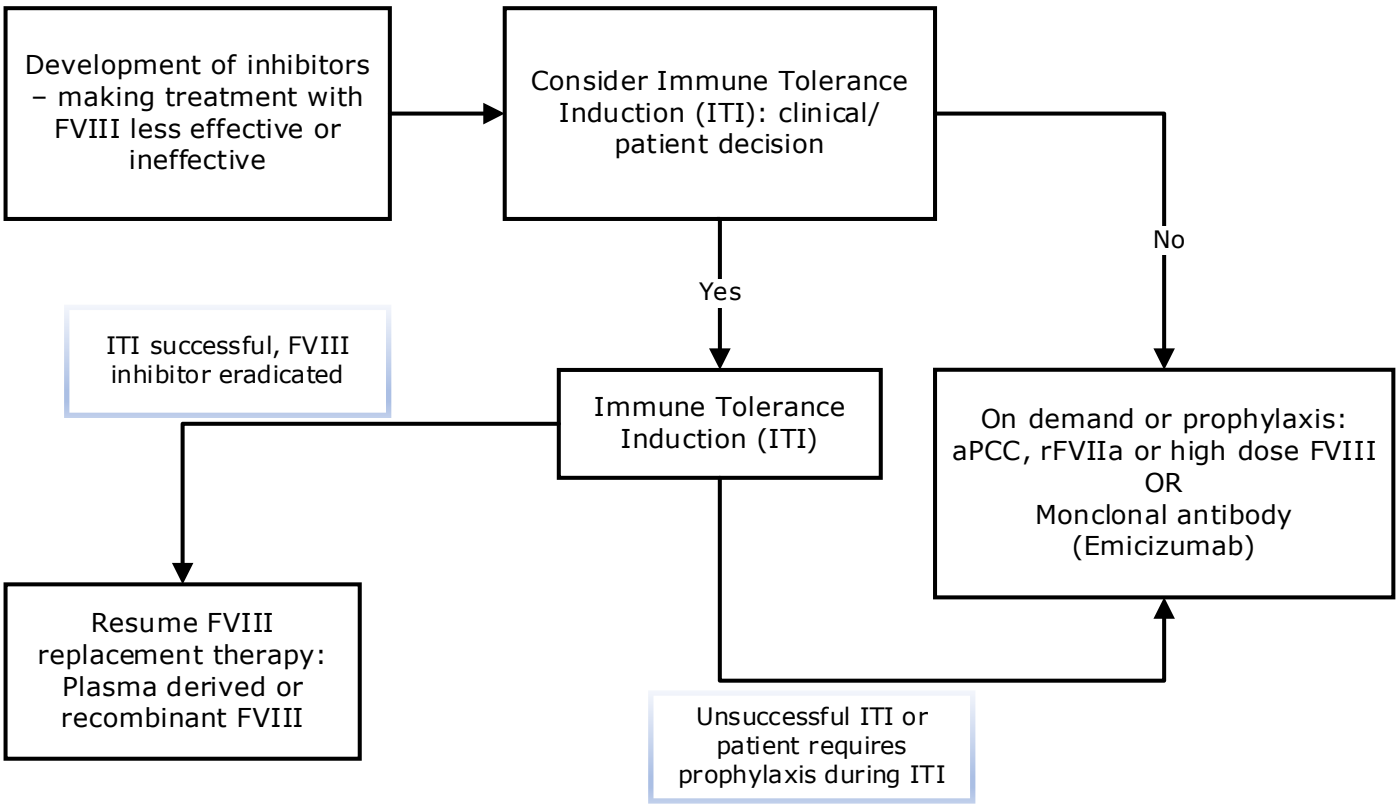
6. Equality Impact and Assessment

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 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.

Annex i Patient Pathway



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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-10	D66	Hereditary Factor VIII deficiency

Annex iii 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.

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