GIG CYMRU NHS WALES
Cyd-bwyllgor Comisiynu
Joint Commissioning Committee

Etranacogene dezaparvovec for treating moderately severe or severe haemophilia B in adults

Policy Position Statement: PPS304

Document Information	
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Target Audience	Chief Executives, Medical Directors, Directors of Finance, Directors of Pharmacy, Haemophilia Consultants, The Haemophilia Society, CSL-Behring, Consultant ATMP Pharmacist
Description	NHS Wales will commission this specialised service in accordance with the criteria described in this policy

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Policy Statement

NHS Wales Joint Commissioning Committee (NWJCC) will commission etranacogene dezaparvovec (Hemgenix®) for adults with moderately severe or severe haemophilia B 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 (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. 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 etranacogene dezaparvovec for treating adults with moderately severe or severe haemophilia B 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 etranacogene dezaparvovec should be made available for treating adults resident in Wales with moderately severe or severe haemophilia B.

Please note that NICE have recommended etranacogene dezaparvovec for treating adults with moderately severe or severe haemophilia B with a managed access agreement[1]. This means that NICE will continue to collect data on the effectiveness of the treatment for subsequent review. After which, NICE will decide whether to recommend it for routine use in the NHS and update the guidance. This policy covers the commissioning of etranacogene dezaparvovec for treating adults with moderately severe or severe haemophilia B for the duration of the managed access agreement.

1.1 Background

Haemophilia B

Haemophilia is a lifelong, inherited condition where the blood doesn't clot properly. This is due to low levels of a protein called factor IX (FIX), which makes it harder to stop bleeding. In moderate and severe cases, bleeding can happen inside joints and muscles without injury, causing pain, stiffness and long-term joint damage[2].

The severity of haemophilia B is classified according to how much clotting factor (FIX) is in the blood. Severe cases have less than 1% of normal levels and moderate cases have 1-5%. Moderately severe haemophilia B does not have a standard definition but is generally considered to have less than 2% of normal clotting factor[3].

Treatment options for managing haemophilia B vary depending on the severity. Generally bleeding is controlled or prevented by replacing the missing clotting factor IX in the blood through an injection. This treatment temporarily increases the FIX levels in

[1] https://www.nice.org.uk/guidance/ta989
[2] https://www.nhs.uk/conditions/haemophilia/
[3] https://haemophilia.org.uk/bleeding-disorders/haemophilia-a-and-b/types-of-haemophilia/

the blood and assists the blood to clot. Newer treatments such as etranacogene dezaparvovec offer the potential for longer-term management[4].

Etranacogene dezaparvovec

Etranacogene dezaparvovec is a type of advanced therapy medicinal product (ATMP) called a gene therapy. Gene therapies work by delivering a gene into the body to correct a genetic defect or by editing a gene to counter the faulty gene. The active substance of etranacogene dezaparvovec is based on a virus (adeno-associated virus) that does not cause disease in humans. This virus has been modified so that it cannot spread in the body but can deliver a functioning copy of the FIX gene into the liver cells. This allows the liver to produce the FIX protein and raise the levels of working FIX in the blood. This helps the blood to clot more normally and prevents or reduces bleeding episodes[5].

Delivery of etranacogene dezaparvovec is managed through Haemophilia Comprehensive Care Centres in a hub and spoke network, in line with UKHCDO guidelines. Treatment is given in an authorised treatment centre (hub) by doctors experienced in the treatment of haemophilia B. The treatment is given as a single slow infusion (drip) into a vein over approximately 1-2 hours. Etranacogene dezaparvovec can only be given once.

Prevalence

Haemophilia B is classified as a rare disease with the prevalence estimated at around 3.8 per 100,000[6]. In 2024/2025, there were 28 adults in Wales living with a factor level less than or equal to 5 IU/dl[7]. This policy relates to a subset of this population who meet the eligibility criteria for treatment.

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).

[4] https://www.england.nhs.uk/blog/welcoming-a-new-era-of-innovative-treatment-for-haemophilia-patients/
[5] https://www.medicines.org.uk/emc/product/14702/pil
[6] Iorio A, et al. Ann Intern Med. 2019;171(8):540-546
[7] Data provided by the National Haemophilia Database – Bleeding Disorder Statistics for Wales 2024-2025.

An EIA was carried out by NICE during the evaluation of etranacogene dezaparvovec and has been adopted for the purposes of implementing this policy. For further details, please refer to the NICE website at: NICE-equality-impact-assessment.

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

2.1 Inclusion Criteria

Etranacogene dezaparvovec is recommended with managed access as an option for treating moderately severe or severe haemophilia B (congenital factor IX [FIX] deficiency) in adults without anti-FIX antibodies. It is only recommended if the conditions of the managed access agreement for etranacogene dezaparvovec are followed[8].

2.2 Continuation of Treatment

Etranacogene dezaparvovec is given as a single dose, with no subsequent doses.

Healthcare professionals are expected to review a patient's health at regular intervals. This will be guided by the clinical team but is typically intensive for the 1st year (at least twice weekly for the first 3 months), and then regular life-long follow up .They should follow national guidelines for patient monitoring and management after gene therapies for haemophilia[9].

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 Designated Providers

Treatment with etranacogene dezaparvovec for the population of Wales will be provided by the following specialist centres:

Cardiff Haemophilia Comprehensive Care Centre
University Hospital Wales
Heath Park Way
Cardiff
CF14 4XW

[8] https://www.nice.org.uk/guidance/ta989/chapter/1-Recommendation
[9] Chowdary P, et al. Haemophilia. 2025;31(1):26-38

Manchester Adult's Haemophilia Comprehensive Care Centre
2nd Floor, Haematology Outpatient Services
Manchester Royal Infirmary
Oxford Road
Manchester
M13 9WL

2.5 Patient Pathway (Annex i)

See Annex i for patient pathway details.

2.6 Mechanism for funding

Etranacogene dezaparvovec 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, the appropriate Blueteq® forms should be completed for approval by the designated provider issuing the treatment. There are three Blueteq® forms associated with etranacogene dezaparvovec:

- Initial Funding Application (submitted as an intention to treat, prior to infusion of etranacogene dezaparvovec)
- Post Infusion Funding Application (submitted to confirm whether the patient received the treatment or not)
- Notification of Treatment Failure (submitted ONLY if treatment failure criteria have been met)

For further information on accessing and completing the Blueteq® form please contact NWJCC using the following email address: NWJCC.Blueteq@wales.nhs.uk.

Funding is approved on the basis that etranacogene dezaparvovec is prescribed and administered in accordance with its marketing authorisation and NICE recommendation (TA989)[1]. The list price of etranacogene dezaparvovec is £2,600,000[10]. The company has a commercial arrangement, which makes etranacogene dezaparvovec 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 a non-contracted provider wishes to treat a patient that meets the criteria they should contact NWJCC at NWJCC.ICP@wales.nhs.uk.

[10] TA989 NICE Final Guidance https://www.nice.org.uk/guidance/ta989/resources/etranacogene-dezaparvovec-for-treating-moderately-severe-or-severe-haemophilia-b-pdf-82615918798789

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.

Gene therapies such as etranacogene dezaparvovec are associated with a long term follow up mandate and will require the regular submission of clinical and quality outcomes to NWJCC by the providers.

Outcomes Reporting Arrangement

An Outcomes Reporting Arrangement has been agreed between the commissioners, NHS Wales Joint Commissioning Committee (NWJCC), and providers for the use of etranacogene dezaparvovec. This arrangement includes, but is not limited to, the submission of clinical outcomes, patient-reported outcome measures, Blueteq forms, and activity data. Providers are expected to submit this data regularly to NWJCC for the agreed period of time.

For further information and the template for submission, please contact NWJCC.Information@wales.nhs.uk.

2.8 Action to be taken

- Providers 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 etranacogene dezaparvovec is purchased at the agreed discounted price.
- Providers are to ensure the need to approve etranacogene dezaparvovec at the appropriate MDT and are registering use on the Blueteq® system. 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.
- The Provider should comply with the reporting arrangements and should regularly provide NWJCC with the appropriate data, for the agreed time period.

3. Listening to People

3.1 Complaints, Incidents and Redress Process

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 Listening to People, The NHS Wales Complaints, Incidents and Redress Process – People's Guidance 2026. 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.

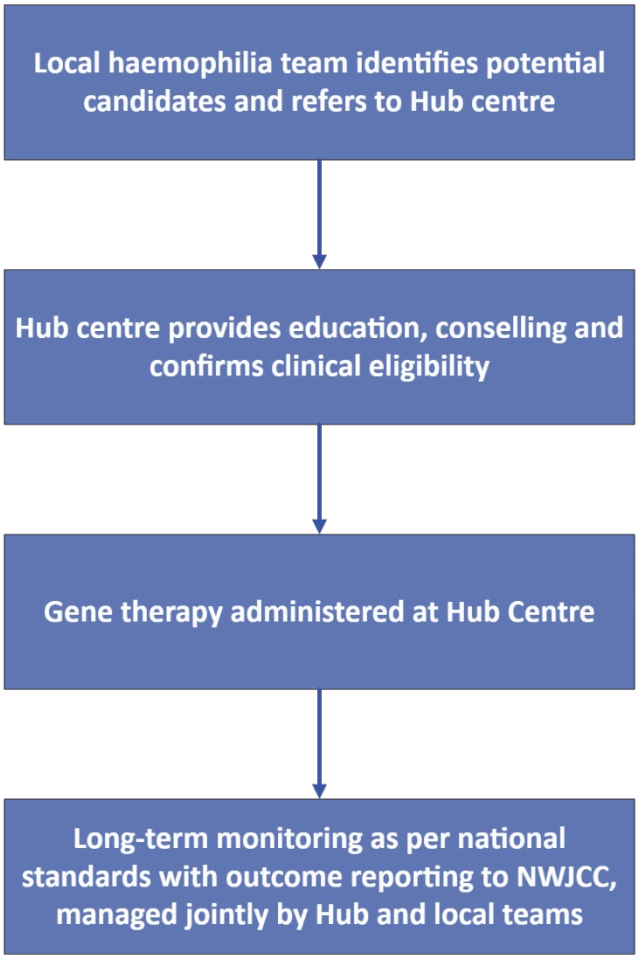
3.2 Individual Patient Funding Request (IPFR)

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 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



Annex ii Codes

The list of ICD codes below is indicative and is not exhaustive. The ICD10 codes have been provided and verified by the Information Standards Team at Digital Health and Care Wales (DCHW). 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	D67	Hereditary Factor IX deficiency

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 – NWJCC.Consultation@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