GIG CYMRU NHS WALES
Cyd-bwyllgor Comisiynu
Joint Commissioning Committee

Efanesoctocog alfa for treating and preventing bleeding episodes in haemophilia A in people 2 years and over

Policy Position Statement: PPS327

Document Information	
Document Name	Efanesoctocog alfa for treating and preventing bleeding episodes in haemophilia A in people 2 years and over
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Target Audience	Chief Executives, Medical Directors, Directors of Finance, Haematology Clinical Leads, Haemophilia Clinical Nurse Specialists, Medicines Manufacturer, Haemophilia Society, Haemophilia Wales
Description	NHS Wales will routinely 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 efanesoctocog alfa for treating and preventing bleeding episodes in people with haemophilia A aged 2 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 (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 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 efanesoctocog alfa 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.

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 efanesoctocog alfa should be made available.

1.1 Background

Haemophilia A is a bleeding disorder which can be categorised as severe, moderate or mild. In the majority of cases haemophilia A is an inherited condition[2]. Our bodies have clotting factors, or proteins, that work together to form a blood clot which stops bleeding[3]. Haemophilia A is caused by a gene mutation that results in the inability or reduced ability to produce a functional clotting factor, factor VIII, which is needed for stable blood clot formation. Haemophilia can lead to prolonged bleeding after injury and in some instances bleeding into joints and muscles without any injury[4]. The treatment needed will depend on the severity of haemophilia. In some cases, treatment is only needed to stop or reduce bleeding when it starts. However, other people may require regular treatment to prevent bleeding[5].

The lower the factor VIII level the higher the risk of bleeding.

Efanesoctocog alfa is a replacement factor VIII therapy. Activated factor VIII helps activated factor IX turn factor X into its active form more quickly. Activated factor X converts prothrombin into thrombin. Thrombin then converts fibrinogen into fibrin and a clot can be formed. Replacement therapy results in an increased level of plasma factor VIII, thereby enabling a temporary correction of the factor deficiency and correction of the bleeding tendencies[6]. Efanesoctocog alfa has the potential to offer extended protection with less frequent dosing in patients with severe haemophilia[7].

[1] Recommendations | Efanesoctocog alfa for treating and preventing bleeding episodes in haemophilia A in people 2 years and over | Guidance | NICE
[2] Haemophilia - Symptoms, diagnosis and treatment | BMJ Best Practice
[3] Hemophilia A: Definition, Symptoms & Treatment
[4] 3 Committee discussion | Efanesoctocog alfa for treating and preventing bleeding episodes in haemophilia A in people 2 years and over | Guidance | NICE
[5] British Journal of Haematology | Wiley Online Library
[6] ALTUVOCT 250 IU Powder and solvent for solution for injection - Summary of Product Characteristics (SmPC) - (emc) | 100565
[7] Efanesoctocog Alfa Half-Life and Clearance Are Independent of von Willebrand Factor in Severe Hemophilia A: A Post Hoc Analysis from Phase 1/2a Studies | Blood | American Society of Hematology

The prevalence of haemophilia A in England is around 0.013% with 24.6% having a severe (Factor VIII activity level of less than 1%) form. Using the NICE eligible population for efanesoctocog alfa in England data, the estimated prevalence in Wales suggests approximately 100 patients would be eligible for treatment[8].

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).

This policy has been subjected to an Equality Impact Assessment.

An EIA was carried out by NICE during the evaluation of efanesoctocog alfa. For further details, please refer to the NICE website at: NICE EQIA.

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.

[8] Resource impact summary report | Tools and resources | Efanesoctocog alfa for treating and preventing bleeding episodes in haemophilia A in people 2 years and over | Guidance | NICE

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

Efanesoctocog alfa is recommended as an option for treating and preventing bleeding episodes in people 2 years and over with haemophilia A (congenital factor VIII deficiency), only if:

- they have a factor VIII activity level of less than 1% (severe haemophilia A)
- the company provides it according to the commercial arrangement.

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.

For the specialised paediatric services it commissions, the JCC will routinely commission treatment up until a patient is 16 years old. The JCC does not commission specialised paediatric services for patients aged 18 years and older. For patients aged 16 or 17 years of age, the JCC will continue to commission ongoing specialised treatment initiated before the patient's 16th birthday and under the ongoing care of a specialised paediatric team.

2.5 Designated Providers

Paediatric Haemophilia Comprehensive Care Centres:

Alder Hey Children's Hospital
Eaton Road
West Derby
Liverpool
L12 2AP

Noah's Ark Children's Hospital for Wales
Heath Park
Cardiff
CF14 4XW

Adult Haemophilia Comprehensive Care Centres:

The Roald Dahl Haemostasis and Thrombosis Centre
Royal Liverpool University Hospital
Liverpool
L7 8XP

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

Haemophilia Treatment Centres:

Abergavenny Haemophilia Centre

Neville Hall Hospital
Brecon Road
Abergavenny
NP7 7EG

Ysbyty Gwynedd
Penrhosgarnedd
Bangor
Gwynedd
LL57 2PW

Swansea Haemophilia Centre
Singleton Hospital
Sketty Lane
Swansea
SA2 8QA

Please note for paediatric patients initiation of treatment and ongoing prescribing must remain within the paediatric haemophilia comprehensive care centres listed.

For adult patients prescribing must be initiated by the adult comprehensive care centres. Continuation of prescribing can be transferred to the haemophilia treatment centres listed if required.

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

Efanesoctocog alfa 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: NWJCC.Blueteq@wales.nhs.uk.

If a non-contracted provider wishes to treat a patient that meets the criteria, they should contact NWJCC.ICP@wales.nhs.uk. They will be asked to demonstrate they have an appropriate MDT in place.

Funding is approved on the basis that efanesoctocog alfa is prescribed and administered in accordance with its marketing authorisation[5]. Efanesoctocog alfa is available as 250 units, 500 units, 1,000 units, 2,000 units, 3,000 units and 4,000 units vials. The cost per vial of 1,000 units efanesoctocog alfa is £2,400 (£2.40 per unit) (excluding VAT; company's evidence submission). The company has a commercial arrangement. This makes efanesoctocog alfa 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.

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 drug will be commissioned.
- NWJCC are to ensure that all providers are purchasing efanesoctocog alfa at the agreed discounted price.
- Providers are to ensure the need to approve efanesoctocog alfa 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.
- Providers are to determine estimated patients numbers and the current dose of any patient(s) who will transfer from any company compassionate use scheme EAMS.
- The provider should work to written quality standards and provide monitoring information to NWJCC on request.

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.

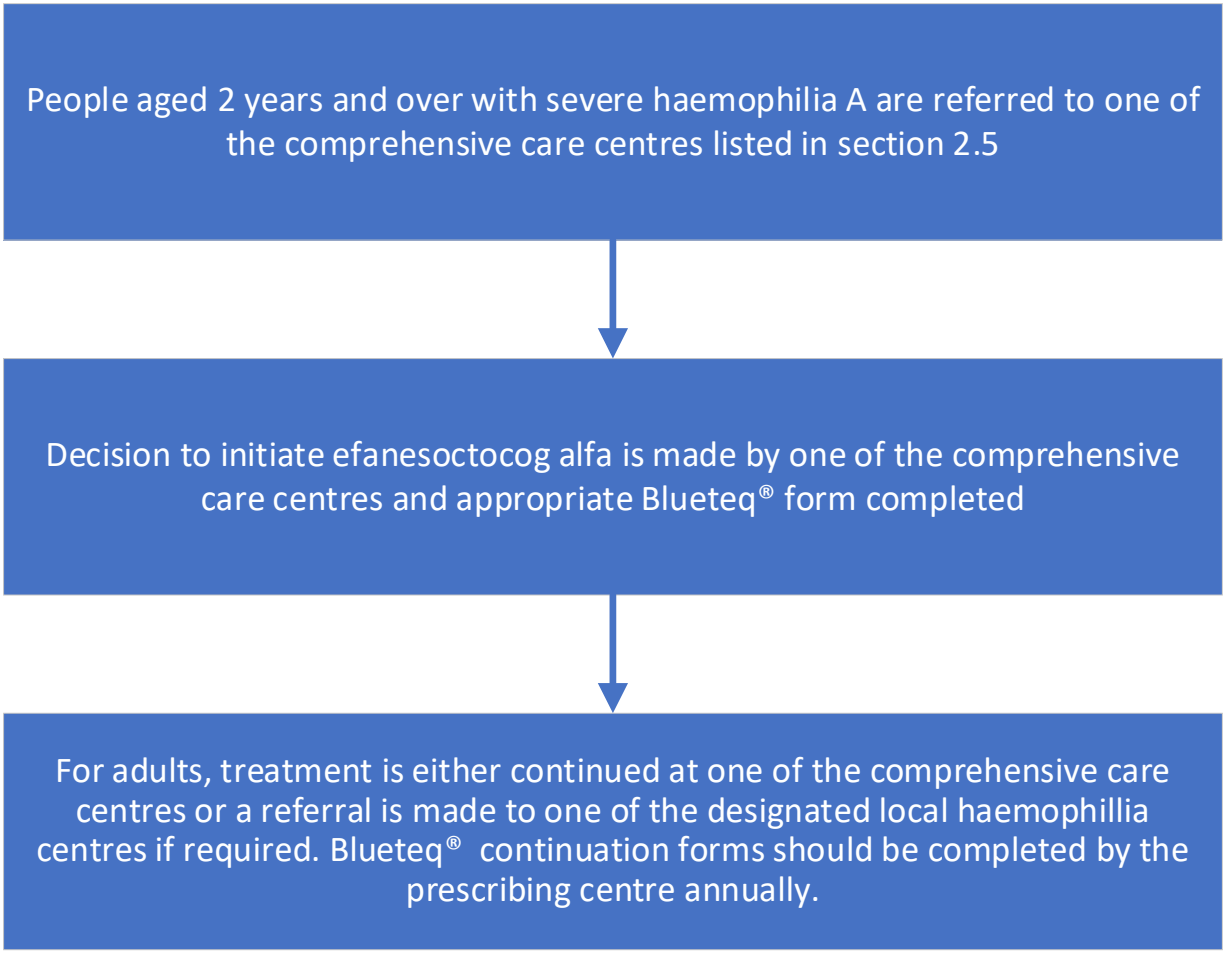
3.2 Individual Patient Funding Request (IPFR)

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	D66.X	**Hereditary factor VIII deficiency** Incl: Deficiency factor VIII (with functional defect) Haemophilia: • NOS • A • classical

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