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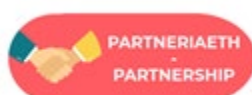
Pwyllgor Gwasanaethau Iechyd
Arbenigol Cymru (PGIAC)
Welsh Health Specialised
Services Committee (WHSSC)

Specialised Services Policy Position PP236

Berotrallstat for preventing recurrent attacks of hereditary angioedema for people aged 12 years and older

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Version 1.0



Document information

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Contents

Policy Statement	4
1. Introduction	5
1.1 Plain language summary	5
1.2 Aims and Objectives	5
1.3 Epidemiology	5
1.4 Current Treatment.....	6
1.5 Proposed Treatment	6
1.6 What NHS Wales has decided.....	6
2. Criteria for Commissioning	7
2.1 Inclusion Criteria	7
2.2 Stopping Criteria	7
2.3 Initiation of Treatment	7
2.4 Continuation of Treatment.....	7
2.5 Acceptance Criteria.....	8
2.6 Exceptions.....	8
2.7 Transition Arrangements	8
2.8 Clinical Outcome and Quality Measures	9
2.9 Responsibilities	9
2.10 Blueteq and reimbursement.....	9
2.11 Action to be taken	10
3. Documents which have informed this policy	11
4. Date of Review	11
5. Putting Things Right.....	12
5.1 Raising a Concern.....	12
5.2 Individual Patient Funding Request (IPFR)	12
6. Equality Impact and Assessment.....	13
Annex i Codes	14
Annex ii Abbreviations and Glossary	15

Policy Statement

Welsh Health Specialised Services Committee (WHSSC) commission berotralstat for people aged 12 years and older with hereditary angioedema in accordance with the criteria outlined in this document.

In creating this document WHSSC has reviewed the relevant guidance issued by National Institute of Health and Care Excellence (NICE)¹ and has concluded that berotralstat should be made available.

Disclaimer

WHSSC assumes that healthcare professionals will use their clinical judgment, knowledge and expertise when deciding whether it is appropriate to apply this policy position statement.

This policy 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.

WHSSC disclaims any responsibility for damages arising out of the use or non-use of this policy position statement.

¹ [Berotralstat for preventing recurrent attacks of hereditary angioedema \[TA738\], October 2021](#)

1. Introduction

This Policy Position Statement has been developed for the planning and delivery of berotralstat for people aged 12 years and older with hereditary angioedema and resident in Wales. This service will only be commissioned by the Welsh Health Specialised Services Committee (WHSSC) and applies to residents of all seven Health Boards in Wales.

1.1 Plain language summary

Hereditary angioedema is a rare genetic disorder. It is a relapsing condition that causes unpredictable and recurrent attacks of swelling. This is usually in the mouth, gut or airway, but it can affect multiple places in the body at once. It often leads to difficulty breathing and severe pain. Acute attacks of hereditary angioedema are difficult to predict and can vary in severity from mild to life threatening. Attacks can significantly affect the quality of life of people with this condition, as well as that of their family members and carers. Attacks can be triggered by anxiety and stress; for example, exams, surgery or dental treatment, as well as positive life events such as weddings and holidays.

Berotralstat is an innovative prophylactic treatment for recurrent attacks of hereditary angioedema. The aim of prophylactic treatment is to reduce the rate and severity of attacks and allow people to live an attack-free life. There are currently no effective licensed oral prophylactic treatments.

1.2 Aims and Objectives

This Policy Position Statement aims to define the commissioning position of WHSSC on the use of berotralstat for people aged 12 years and older with hereditary angioedema.

The objectives of this policy are to:

- ensure commissioning for the use of berotralstat is evidence based
- ensure equitable access to berotralstat
- define criteria for people with hereditary angioedema to access treatment
- improve outcomes for people with hereditary angioedema.

1.3 Epidemiology

Hereditary angioedema affects approximately 1 per 10,000 to 50,000 people, and usually develops in the first 10 to 20 years of life².

² <https://www.nice.org.uk/guidance/ta738/>

1.4 Current Treatment

There are not many effective treatments available for preventing recurrent attacks of hereditary angioedema. Attacks are usually treated as they happen. The frequency and severity of attacks of angioedema can be prophylactically reduced by attenuated androgens such as Danazol and Oxandrolone, or by Tranexamic Acid but these can cause unwanted side effects and require close monitoring. Oral agents used in Wales are Danazol and tranexamic acid with stanozalol and oxandrolone use being less common.

1.5 Proposed Treatment

Berotralstat (Orladeyo®) is indicated for 'routine prevention of recurrent attacks of hereditary angioedema (HAE) in adult and adolescent patients aged 12 years and older'.

1.6 What NHS Wales has decided

WHSSC has carefully reviewed the relevant guidance issued by National Institute of Health and Care Excellence (NICE). We have concluded that berotralstat, should be made available within the criteria set out in section 2.1.

2. Criteria for Commissioning

The Welsh Health Specialised Services Committee approve funding of berotralstat for people aged 12 and older with hereditary angioedema in line with the criteria identified in the policy.

2.1 Inclusion Criteria

- Berotralstat is recommended as an option for preventing recurrent attacks of hereditary angioedema in people 12 years and older, only if:
 - they have at least 2 attacks per month, and
 - it is stopped if the number of attacks per month does not reduce by at least 50% after 3 months.
- It is only recommended if the company provides berotralstat according to the commercial arrangement³.
- This recommendation is not intended to affect treatment with berotralstat 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 clinician consider it appropriate to stop. For young people, this decision should be made jointly by the clinician and the young person and the young person's parents or carers.

2.2 Stopping Criteria

Berotralstat should be stopped if the number of attacks per month does not reduce by at least 50% after 3 months.

2.3 Initiation of Treatment

Berotralstat should be initiated only by consultant immunologists experienced in the care of patients with HAE.

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.

³ **Commercial arrangement:** There is a simple discount patient access scheme for berotralstat. Contact enquiries@biocryst.co.uk for details.

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 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, and IPFR should be submitted.

Further information on making IPFR requests can be found at: [Welsh Health Specialised Services Committee \(WHSSC\) | Individual Patient Funding Requests](#)

2.7 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.

2.8 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 Responsibilities

Referrers should:

- inform the patient that this treatment is not routinely funded outside the criteria in this policy, and

Clinicians considering treatment should:

- discuss all the alternative treatments with the patient
- advise the patient of any side effects and risks of the potential treatment
- inform the patient that treatment is not routinely funded outside of the criteria in the policy, and
- confirm that there is contractual agreement with WHSSC for the treatment.

In all other circumstances an IPFR must be submitted.

2.10 Blueteq and reimbursement

Berotralstat for preventing recurrent attacks of hereditary angioedema will only be funded for patients registered via the Blueteq system and where treatment is initiated by a consultant immunologists experienced in the care of patients with HAE.

Where the patient meet 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 WHSSC using the following e-mail address: WHSSC.blueteq@wales.nhs.uk

If a non-contracted provider wishes to treat a patient that meets the criteria they should contact WHSSC (e-mail: WHSSC.blueteq@wales.nhs.uk). They will be asked to demonstrate they have an appropriate MDT in place.

Funding is approved on the basis that berotralstat is prescribed and administered in accordance with its Marketing Authorisation⁴.

⁴ [Orladeyo - Summary of Product Characteristics \(SmPC\) - \(emc\) \(medicines.org.uk\)](#)

The list price of berotralstat is £10,205 for a 28-pack of 150 mg capsules (company submission), which equates to an annual cost of £133,120.60. The company has a commercial arrangement. This makes berotralstat available to the NHS with a discount. The size of the discount is commercial in confidence. Health Boards in Wales should refer to the AWTTTC Vault for further information on the Patient Access Scheme (PAS) price.

2.11 Action to be taken

- Health Boards are to circulate this Policy Position Statement to all Hospitals/MDTs to inform them of the conditions under which the technology will be commissioned.
- Health Boards are to ensure that all providers are purchasing berotralstat at the agreed discounted price.
- Health Boards are to ensure that they 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 WHSSC on request.

3. Documents which have informed this policy

The following documents have been used to inform this policy:

- **National Institute of Health and Care Excellence (NICE) guidance**
 - [Berotralstat for preventing recurrent attacks of hereditary angioedema](#), NICE Technology appraisal guidance (TA738), October 2021

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

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

4. Date of Review

This document will be reviewed when information is received which indicates that the policy requires revision.

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 WHSSC.

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.

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, an IPFR should be submitted.

Further information on making IPFR requests can be found at: [Welsh Health Specialised Services Committee \(WHSSC\) | 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 Welsh Health Specialised Services 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 Codes

Code Category	Code	Description
ICD-10	D841	Hereditary angioedema (disorder)

Annex ii Abbreviations and Glossary

Abbreviations

HAE	Hereditary Angioedema
IPFR	Individual Patient Funding Request
NICE	National Institute of Health and Care Excellence
WHSSC	Welsh Health Specialised Services Committee

Glossary

Individual Patient Funding Request (IPFR)

An IPFR is a request to Welsh Health Specialised Services Committee (WHSSC) to fund an intervention, device or treatment for patients that fall outside the range of services and treatments routinely provided across Wales.

Welsh Health Specialised Services Committee (WHSSC)

WHSSC is a joint committee of the seven local health boards in Wales. The purpose of WHSSC is to ensure that the population of Wales has fair and equitable access to the full range of Specialised Services and Tertiary Services. WHSSC ensures that specialised services 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.