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Drug treatments for atypical Haemolytic Uraemic Syndrome (aHUS)

Policy Position Statement: PPS260

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Policy Statement

NHS Wales Joint Commissioning Committee (NWJCC) commission both eculizumab¹ and ravulizumab² for people with atypical haemolytic uraemic syndrome (aHUS) 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. 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.

¹ [Overview | Eculizumab for treating atypical haemolytic uraemic syndrome | Guidance | NICE](#)

² [Overview | Ravulizumab for treating atypical haemolytic uraemic syndrome | Guidance | NICE](#)

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 eculizumab and ravulizumab for aHUS 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, 2} and has concluded eculizumab and ravulizumab should be made available.

1.1 Background

Atypical haemolytic uraemic syndrome is a rare genetic and progressive disease caused by a fault in the complement system³. The complement system is part of the body's immune response that attacks microbes. The body usually has an in-built system of protector proteins that stop complement from attacking the body's own cells. In aHUS, complement starts to attack the body's own cells, especially cells that line the blood vessels. This leads to clots forming within the small vessels. The most commonly affected organ is the kidney but all organs can be affected. Left untreated it can be a life-threatening illness and the majority of people would develop end stage kidney failure and would require long term dialysis.

The first sign of aHUS is usually increasing tiredness and generally feeling unwell. Other symptoms may include⁴:

- headache
- blurred vision
- nausea and/or vomiting
- stomach pain
- chest pain
- rash
- ulcers
- blood in the urine (haematuria)
- protein in the urine (proteinuria)

These symptoms may be mild at first but worsen over time.

An aHUS episode can occur at any age and can be triggered by⁴:

³ [What is AHUS? - Atypical Hus](#)

⁴ [Atypical Haemolytic Uraemic Syndrome \(aHUS\) | Kidney Care UK](#)

- gastrointestinal illness – vomiting and/or diarrhoea
- respiratory illness – a cold or flu
- certain medications
- pregnancy – especially in the third trimester and post-delivery

Initially aHUS is diagnosed by blood and urine tests and by ruling out other similar conditions such as typical haemolytic-uremic syndrome⁴. The two disorders have different causes and different signs and symptoms. Tests for aHUS may include:

- Full blood count – to examine the haemoglobin (red blood cell) and platelet levels in the blood
- Complement studies – to measure the amount of certain proteins in the blood
- Urinalysis – to check for blood or protein in the urine, which may be a sign of kidney damage
- Stool/rectal swab – to exclude ‘typical’ HUS, also known as STEC HUS or diarrhoea positive HUS

In some cases, a kidney biopsy and/or an ultrasound is performed to check the function and structure of the kidneys. Genetic testing will then be done through specialised centre(s) to confirm aHUS⁵.

Without treatment, aHUS can lead to end stage kidney failure. A short-term course of dialysis may be needed to improve kidney function. If renal damage is severe, lifelong dialysis may be needed. A transplant may be offered, but there is a risk that aHUS can reoccur in the newly transplanted kidney as the faulty complement proteins are still produced by the liver^{5, 6}. Historically treatment involved plasma exchange, a process by which the plasma that contains the faulty complement proteins is removed from the blood and replaced with fresh plasma from donated blood that contains functioning proteins⁵.

Eculizumab and ravulizumab are complement C5 inhibitors^{6,7}. They work by blocking the action of complement proteins, thereby preventing their pro inflammatory and pro thrombotic effects which damages the body’s own cells⁵.

There is an incidence of around 20 new cases of aHUS in the UK each year⁵. Extrapolating for the Welsh population that equates to around 1 new case per year in Wales.

⁵ [Diagnosis – aHUS Foundation](#)

⁶ [Eculizumab | Drugs | BNF | NICE](#)

⁷ [Ravulizumab | Drugs | BNF | NICE](#)

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 EIA was carried out by NICE during the evaluation of eculizumab and ravulizumab. For further details, please refer to the NICE website for the relevant EQIA's: [EQIA-eculizumab](#) and [EQIA-ravulizumab](#).

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 for eculizumab¹ and ravulizumab², alongside the individual needs, preferences and values of the patient.

2.1 Inclusion Criteria

Eculizumab for treating atypical haemolytic uraemic syndrome (HST1)

Eculizumab, within its marketing authorisation, is recommended for funding for treating atypical haemolytic uraemic syndrome, only if all the following arrangements are in place:

- coordination of eculizumab use through an expert centre
- monitoring systems to record the number of people with a diagnosis of atypical haemolytic uraemic syndrome and the number who have eculizumab, and the dose and duration of treatment
- a national protocol for starting and stopping eculizumab for clinical reasons
- a research programme with robust methods to evaluate when stopping treatment or dose adjustment might occur¹.

Ravulizumab for treating atypical haemolytic uraemic syndrome (TA710)

Ravulizumab is recommended, within its marketing authorisation, as an option for treating atypical haemolytic uraemic syndrome (aHUS) in people weighing 10 kg or more:

- who have not had a complement inhibitor before or
- whose disease has responded to at least 3 months of eculizumab treatment.

It is recommended only if the company provides ravulizumab according to the commercial arrangement².

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

Adult Service Providers

University Hospital of Wales (UHW)

Heath Park
Caerdydd
CF14 4XW

Morrison Hospital

Heol Maes yr Eglwys
Cwmrhydyceirw
Abertawe
SA6 6NL

Ysbyty Gwynedd

Penrhosgarnedd
Bangor
LL57 2PW

Ysbyty Glan Clwyd

Rhuddlan Rd
Bodelwyddan
Rhyl
LL18 5UJ

Wrexham Maelor Hospital

Croesnewydd Rd
Wrexham
LL13 7TD

Paediatric Service Providers

Childrens Hospital for Wales

University Hospital of Wales (UHW)
Heath Park
Caerdydd
CF14 4XW

Alder Hey Children's Hospital

Eaton Road
Liverpool
L12 2AP

2.6 Patient Pathway (Annex i)

Please see Annex i.

2.7 Mechanism for funding

Eculizumab and ravulizumab will only be funded for patients registered via the Blueteq[®] system. The Blueteq[®] forms can be completed by any designated provider listed in section 2.5 of the policy as long as approval via the National Renal Complement Therapeutics Centre in Newcastle has been secured.

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

Funding is approved on the basis that eculizumab and ravulizumab is prescribed and administered in accordance with their marketing authorisations respectively.

The companies have commercial arrangements. This makes eculizumab and ravulizumab 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 Commercial Medicines Access Reference Tool (CMART) for further information on the Patient Access Scheme (PAS) price.

Where biosimilar products are available, the provider will only be reimbursed for the price of the best value biosimilar product. In the instance of a biosimilar not being prescribed the provider must notify NWJCC by using the following link: Individual Patient Funding Requests

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 technology will be commissioned.
- Providers are to ensure that they are purchasing eculizumab and ravulizumab at the agreed discounted price.
- Providers are to ensure that eculizumab and ravulizumab are approved at the appropriate MDT and use is registered on the Blueteq system, and the treatment will only be funded where the Blueteq® minimum dataset is fully and accurately populated.
- Providers 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.

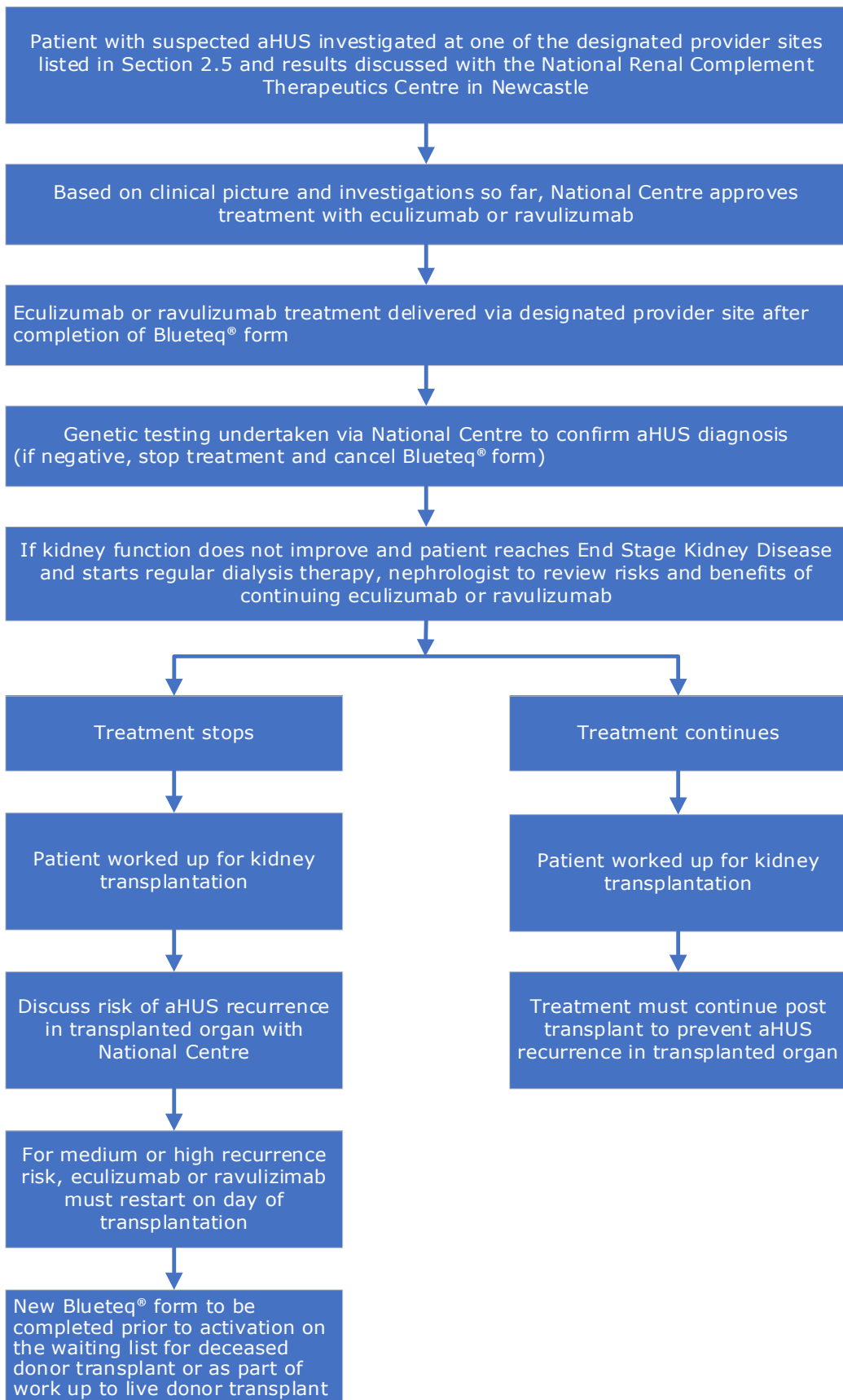
3.2 Individual Patient Funding Request (IPFR)

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.

If the patient wishes to be referred to a provider outside of the agreed pathway, and an IPFR should be submitted.

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