GIG CYMRU NHS WALES

Pwyllgor Gwasanaethau Iechyd Arbenigol Cymru (PGIAC)

Welsh Health Specialised Services Committee (WHSSC)

Specialised Services
Policy Position Statement PP252

Givosiran for treating acute hepatic porphyria for people aged 12 years and older

April 2023

Version 0.5

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Target audience	**For information** Chief Executives, Medical Directors, Directors of Finance, Directors of Planning, Director of Nursing Planning Managers
	For action Chief Pharmacists, Clinical Lead Metabolic Medicine Director of Operations for Specialist Services, Directorate Manager for Metabolic Medicine, Specialist Head of Finance and Commissioning, Health Board Commissioning Managers,
Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy
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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.

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

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1. Introduction

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

In creating this document WHSSC has reviewed the relevant guidance issued by the National Institute of Health and Care Excellence (NICE)[1] and has concluded that givosiran for treating acute hepatic porphyria should be made available.

Welsh Language

WHSSC 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 WHSSC 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

WHSSC 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 WHSSC commitment.

1.1 Background

Acute hepatic porphyria (AHP) is a rare inherited metabolic disorder caused by a deficiency of the enzyme needed to make haem (a substance that gives blood its red colour). It is characterised by high levels of chemical know as porphyrin precursors, in the liver and other tissues. High levels of

[1] Overview | Givosiran for treating acute hepatic porphyria | Guidance | NICE

these substances damage nerve cells and can provoke acute attacks of physical pain.

AHP is a progressive and potentially life-threatening condition that can significantly affect the quality of life of people with the condition, and their families and carers. People can have acute attacks with extreme pain, nausea and fatigue, which sometimes lead to seizures and paralysis. They can also have chronic pain and fatigue. Acute attacks are very rare before puberty and usually start between age 15 and 35. They are more common in women, who may be at increased risk of having an acute attack during or after pregnancy.

1.2 Epidemiology

The condition varies from person to person. There are 4 types of AHP:

- acute intermittent porphyria
- hereditary coproporphyria
- variegate porphyria
- aminolevulinate dehydratase porphyria.

Acute intermittent porphyria is the most common form of AHP in the UK and has the highest symptom burden. The prevalence of symptomatic AHP is estimated to be 1 in 100,000 people in Europe, which equates to about 30 people in Wales. Most people recover after 1 attack or a few attacks, but attacks can be recurrent in about 10% of people. People with recurrent severe attacks often have chronic symptoms and may not fully recover from an attack. According to the National Acute Porphyria Service, there are 27 people in the UK having treatment for recurrent severe attacks.

1.3 Current treatment

Treatment options for AHP aim to prevent attacks or manage symptoms. They include pain management, stopping medication that could have triggered symptoms, gonadotrophin releasing hormone (GnRH) analogues for hormone-induced attacks in women, and oral or intravenous glucose for acute attacks. Haem arginate is indicated for treating acute attacks of AHP. It is also used outside its marketing authorisation to prevent attacks. Liver transplant may be an option for some people with recurrent severe attacks when other treatment options have not worked.

Givosiran

Givosiran is a medicine used for the treatment of adults with AHP. It is a small-interfering ribonucleic acid (siRNA) that suppresses delta-aminolevulinic acid synthase 1 (ALAS 1) an important enzyme in the production of haem. This reduces the level of toxic precursors of porphyrin.

1.4 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 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 subject to an Equality Impact Assessment in line with guidance contained in Corp-026[2].

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.

An EQIA was also carried out by NICE during the evaluation of Givosiran for treating acute hepatic porphyria. For further details, please refer to the NICE website at: Overview | Givosiran for treating acute hepatic porphyria | Guidance | NICE

[2] Equality Impact Assessment Policy (EQIA), WHSSC Corporate Policy Corp-026 (2022)

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

2.1 Inclusion Criteria

Givosiran is recommended as an option for treating acute hepatic porphyria (AHP) in adults and young people aged 12 and older, only if:

- they have clinically confirmed severe recurrent attacks (4 attacks or more within 12 months) and
- the company provides it according to the commercial arrangement[3].

This recommendation is not intended to affect treatment with givosiran that was started in the NHS before this guidance was published. Adults and young 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. This decision should be made jointly by the clinician, the young person and their parents or carers.

Therapy should be initiated under the supervision of a healthcare professional experienced in the management of porphyria[4].

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.

[3] Overview | Givosiran for treating acute hepatic porphyria | Guidance | NICE
[4] Givlaari 189 mg/mL solution for injection - Summary of Product Characteristics (SmPC) - (emc) (medicines.org.uk)

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

Givosiran treatment will be initiated and monitored in one of the two National Acute Porphyria service (NAPS) providers:

- Cardiff and Vale University Health (CVUHB)
  University Hospital Wales,
  Heath Park,
  Cardiff,
  CF144XW

- King's College Hospital NHS Foundation Trust (KCH)
  Denmark Hill,
  London,
  SE5 9RS

2.6 Patient Pathway (See Annex i)

See Annex i

2.7 Blueteq and reimbursement

Givosiran will only be funded for patients registered via the Blueteq system and where an appropriately constructed MDT has approved its use within the National Acute Porphyria Service.

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

Givosiran has a marketing authorisation in the UK for 'treating acute hepatic porphyria in adults and adolescents aged 12 years or older'. It is administered by subcutaneous injection. It will only be commissioned by WHSSC when prescribed in accordance with its marketing authorisation[5].

The price for givosiran is £41,884.43 per 189 mg vial (excluding VAT; company's evidence submission). The company has a commercial arrangement. This makes givosiran available to the NHS with a discount. The size of the discount is commercial in confidence. It is the company's responsibility to let relevant NHS organisations know details of the discount.

2.8 Action to be taken

- Health Boards and WHSSC are to circulate this Policy Position Statement to all Hospitals/MDTs to inform them of the conditions under which the technology will be commissioned.
- WHSSC are to ensure that all providers are purchasing givosiran at the agreed discounted price.
- Providers are to ensure the need to approve givosiran at the appropriate MDT and are registering use on the Blueteq system. This treatment will only be funded where the Blueteq minimum dataset is fully and accurately populated.
- Providers are to determine estimated patient numbers and the current dose of any patient(s) who will transfer from any company compassionate use scheme or EAMS.
- The Provider should work to written quality standards and provide monitoring information to WHSSC on request.

[5] Givlaari 189 mg/mL solution for injection - Summary of Product Characteristics (SmPC) - (emc) (medicines.org.uk)

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 WHSSC.

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 All Wales IPFR Panel will then consider the request.

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

Annex i Patient Pathway