GIG CYMRU NHS WALES
Cyd-bwyllgor Comisiynu
Joint Commissioning Committee

Treatment options for transthyretin amyloidosis in adults

Policy Position Statement: PPS187

Document Information	
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Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy
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Contents

Policy Statement

NHS Wales Joint Commissioning Committee (NWJCC) will commission patisiran, vutrisiran and eplontersen for adults with stage 1 and stage 2 polyneuropathy with hereditary transthyretin amyloidosis 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 patisiran, vutrisiran and eplontersen for stage 1 and stage 2 polyneuropathy in adults with hereditary transthyretin amyloidosis 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, patisiran, vutrisiran and eplontersen should be made available.

1.1 Background

Hereditary transthyretin (hATTR) amyloidosis

Hereditary transthyretin (hATTR) amyloidosis is an ultra-rare condition caused by inherited mutations in the transthyretin (TTR) gene. There are over 130 variants, mutations that are known to cause hATTR amyloidosis[2]. The mutation causes the liver to produce abnormal TTR proteins which accumulates as deposits in body tissues (amyloidosis). These deposits can disrupt the structure and damage the function of affected tissues.

Hereditary transthyretin (hATTR) amyloidosis can affect tissues throughout the body, and people may have a range of symptoms relating to 1 or more systems. The affected systems include the following:

- autonomic nervous system
- peripheral nerves
- heart
- gastrointestinal system
- eyes
- the central nervous system.

Even though people are born with hATTR, symptoms do not begin appearing until adulthood (usually after the age of 50, although it can be earlier in some people)[2,3].The effects and complications of the condition can lead to death within 3 to 15 years of symptoms developing.

[1] https://www.nice.org.uk/guidance
[2] Types of Amyloidosis - Amyloidosis UK
[3] Hereditary Amyloidosis | Amyloidosis Foundation

The prevalence of hATTR amyloidosis is estimated to be less than 1 in 100,000 people in the general European population. In the UK there are thought to be around 150 people with the disease[4]. This equates to around 7 people in Wales.

1.2 Current Treatment

Current treatment options for hereditary transthyretin amyloidosis are limited and focus on supportive care, symptom relief such as pain management and nutritional and mobility support.

Patisiran
Patisiran is a ribonucleic acid interference agent that prevents TTR production by the liver (including abnormal TTR)[5].

Vutrisiran
Vutrisiran is a ribonucleic acid interference agent that prevents TTR production by the liver (including abnormal TTR)[6].

Eplontersen
Eplontersen is an antisense oligonucleotide inhibitor which prevents TTR production[7].

1.3 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. 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 EIA was carried out by NICE during the evaluation of patisiran, vutrisiran and eplontersen. For further details, please refer to the NICE website at:

- Patisiran: equality-impact-assessment-guidance-development
- Vutrisiran: equality-impact-assessment-guidance-development
- Eplontersen: equality-impact-assessment-guidance-development

[4] NATIONAL INSTITUTE FOR CLINICAL EXCELLENCE
[5] Patisiran | Drugs | BNF | NICE
[6] Vutrisiran | Drugs | BNF | NICE
[7] Eplontersen | Drugs | BNF | 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, alongside the individual needs, preferences and values of the patient.

2.1 Inclusion Criteria

Patisiran

Patisiran is recommended, within its marketing authorisation, as an option for treating hereditary transthyretin amyloidosis in adults with stage 1 and stage 2 polyneuropathy. It is recommended only if the company provides patisiran according to the commercial arrangement[8].

Vutrisiran

Vutrisiran is recommended, within its marketing authorisation, as an option for treating hereditary transthyretin amyloidosis in adults with stage 1 and stage 2 polyneuropathy. It is recommended only if the company provides vutrisiran according to the commercial arrangement[9].

If people with the condition and their clinicians consider vutrisiran to be 1 of a range of suitable treatments, discuss the advantages and disadvantages of the available treatments. After that discussion, if more than 1 treatment is suitable, choose the least expensive. Take account of administration costs, dosage, price per dose and commercial arrangements.

Eplontersen

Eplontersen is recommended, within its marketing authorisation, as an option for treating hereditary transthyretin-related amyloidosis in adults with stage 1 or stage 2 polyneuropathy. It is only recommended if the company provides eplontersen according to the commercial arrangement[10].

Use the least expensive option of the available treatments (including eplontersen and vutrisiran). Take account of administration costs, dosages, price per dose and commercial arrangements. If the least expensive option is unsuitable, people with the condition and their healthcare professional should discuss the advantages and disadvantages of other treatments. Please refer to the full NICE guidance for more information regarding cost comparison.

[8] Overview | Patisiran for treating hereditary transthyretin amyloidosis | Guidance | NICE
[9] Overview | Vutrisiran for treating hereditary transthyretin-related amyloidosis | Guidance | NICE
[10] Overview | Eplontersen for treating hereditary transthyretin-related amyloidosis | Guidance | NICE

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 Designated Providers

NHS National Amyloidosis Centre
Centre for Amyloidosis & Acute Phase Proteins
Division of Medicine (Royal Free Campus)
University College London
Rowland Hill Street
London NW3 2PF

2.5 Patient Pathway (Annex i)

See Annex i.

2.6 Mechanism for funding

Patisiran, vutrisiran and eplontersen 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 at NWJCC.IPC@wales.nhs.uk. They will be asked to demonstrate they have an appropriate MDT in place.

Funding is approved on the basis that the drugs listed in this policy are prescribed and administered in accordance with their marketing authorisation.

The pharmaceutical companies manufacturing patisiran, vutrisiran and eplontersen all have a commercial arrangement in place. This makes the drugs 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.

2.7 Clinical Outcome and Quality Measures

The Provider must work to written quality standards and provide monitoring information to the lead commissioner. (These standards can include clinical outcomes, PROMS, Quality of Life etc).

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.

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 policy will be commissioned.
- Providers are to ensure they are purchasing the drugs listed in this policy at the agreed discounted price.
- Providers are to ensure the need to approve the drugs listed in this policy 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.
- 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.

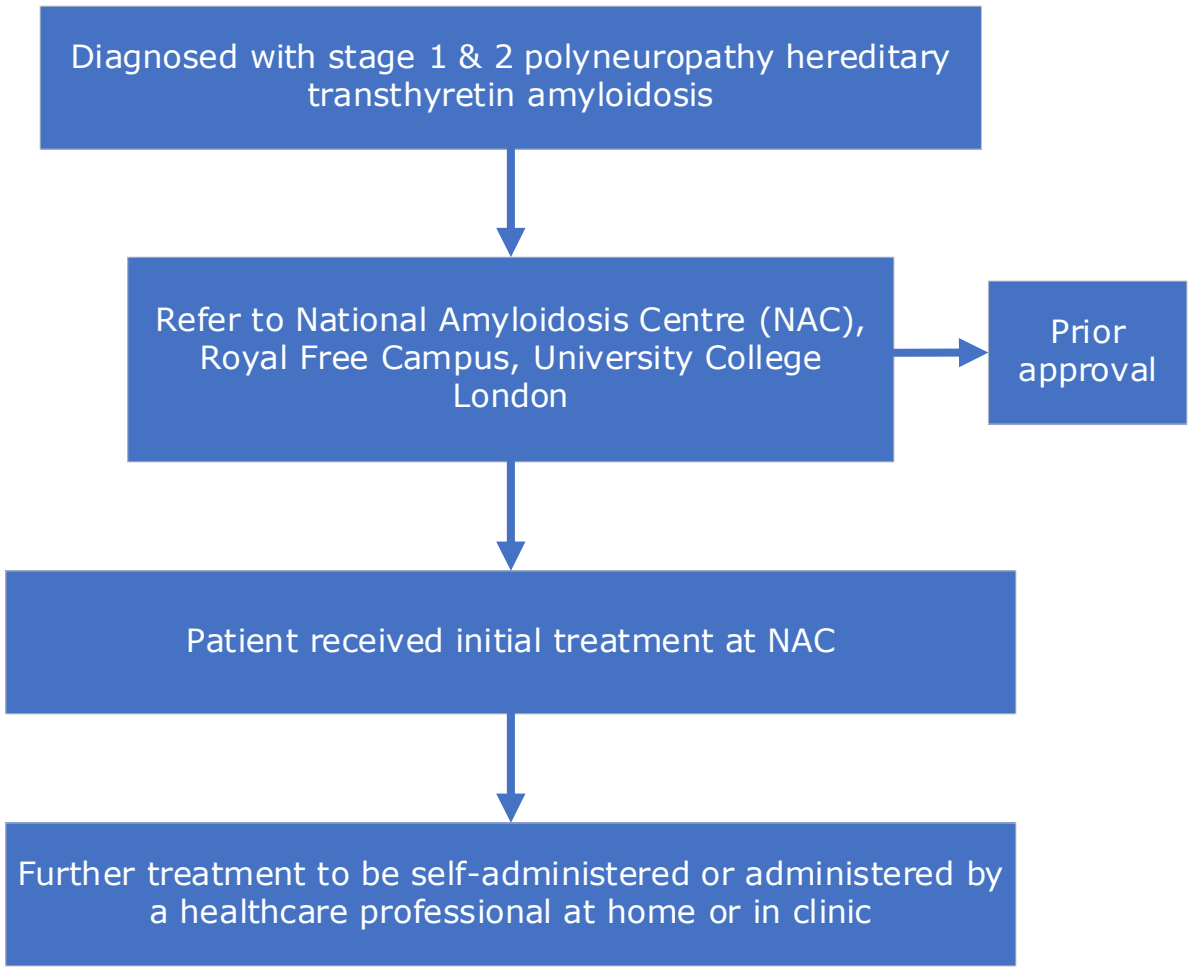
If an IPFR is declined by the Panel, a patient and/or their NHS clinician has the right to request information about how the decision was reached. If the patient and their NHS clinician feel the process has not been followed in accordance with this policy, arrangements can be made for an independent 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, and IPFR should be submitted.

Further information on making IPFR requests can be found at: Individual Patient Funding Requests

Annex i Patient Pathway

Pathway for adults with stage 1 and stage 2 polyneuropathy



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	E85.1	Neuropathic heredofamilial amyloidosis

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