GIG CYMRU NHS WALES
Cyd-bwyllgor Comisiynu
Joint Commissioning Committee

Tafamidis for treating transthyretin amyloidosis with cardiomyopathy

Policy Position Statement: PPS306

Document Information	
Document Name	Tafamidis for treating transthyretin amyloidosis with cardiomyopathy
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Target Audience	Chief Executives, Medical Directors, Directors of Finance, Cardiology Clinicians, National Amyloidosis Centre, Directors of Pharmacy
Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy

Contents

Policy Statement

NHS Wales Joint Commissioning Committee (NWJCC) will commission tafamidis for people with transthyretin amyloidosis with cardiomyopathy 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 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

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 tafamidis for treating transthyretin amyloidosis with cardiomyopathy 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 the tafamidis should be made available.

1.1 Background

Transthyretin amyloidosis is a rare condition in which the body produces abnormal transthyretin amyloid protein[2]. This abnormal insoluble protein enters the circulation and accumulates in various tissues and/or organs throughout the body. These deposits can disrupt or damage the structure and function of the affected areas. This means that patients with transthyretin amyloidosis have a range of symptoms that may affect one or multiple parts of the body. This policy specifically relates to transthyretin amyloidosis with cardiomyopathy (affecting the heart).

Transthyretin amyloidosis with cardiomyopathy, occurs when abnormal transthyretin amyloid protein builds up in the heart muscle (myocardium)[3] and causes restrictive cardiomyopathy[4]. These amyloid deposits make the walls of the heart stiffer. This means that the chambers of the heart cannot squeeze or relax properly making it more difficult to effectively pump blood around the body. As the condition progresses, and pumping becomes more difficult, it can ultimately result in heart failure. There are two types of transthyretin amyloid cardiomyopathies, hereditary and wild-type[5].

- **Hereditary transthyretin amyloid cardiomyopathy**: This is a genetic condition caused by inherited mutations in the transthyretin gene[2]. The mutation causes the liver to produce abnormal transthyretin amyloid protein. This makes the protein more unstable and more likely to form amyloid deposits. This type normally affects the heart and nerves, both the peripheral (hands and feet) and the main nerves[5].
- **Wild type transthyretin amyloid cardiomyopathy**: This does not have a genetic component and is a condition of older age, where normal transthyretin protein becomes unstable and deposited in the heart more gradually[5]. It was previously called 'senile systemic amyloidosis'.

[1] Overview | Tafamidis for treating transthyretin amyloidosis with cardiomyopathy | Guidance | NICE
[2] whssc.nhs.wales/commissioning/whssc-policies/all-policy-documents/treatment-options-for-transthyretin-amyloidosis-in-adults-policy-position-statement-pp187-september-2023/
[3] Transthyretin Amyloid Cardiomyopathy (ATTR-CM) | American Heart Association
[4] Transthyretin Amyloid Cardiomyopathy (ATTR-CM) - National Library of Medicine - NCBI Bookshelf (nih.gov)
[5] Cardiac amyloidosis | Cardiomyopathy UK

Symptoms of hereditary transthyretin amyloid cardiomyopathy can vary and the condition is often misdiagnosed[4]. In early stages, it may mimic the symptoms of other conditions, such as heart failure (shortness of breath at rest or with minimal exertion, swelling of the feet and/or ankles and chest congestion) related to high blood pressure, and enlargement and thickening of the heart (hypertrophic cardiomyopathy). Some people may have no symptoms, while others may progress to end-stage heart failure. The symptoms of wild-type transthyretin amyloid cardiomyopathy are similar but may be mild and remain undiagnosed.

Transthyretin amyloid cardiomyopathy may be suspected because of symptoms such as shortness of breath or swelling of the feet and ankles, leading to routine cardiac tests such as an electrocardiogram and echocardiogram[4]. Once suspected, more specialised tests are needed to confirm the diagnosis. These could include the following tests but see Annex ii for a more comprehensive list:

- Imaging studies of the heart (cardiac MRI)
- A tissue biopsy
- Genetic testing

Some treatments aim to reduce the symptoms associated with transthyretin amyloidosis with cardiomyopathy, including heart failure. Treatments can include diuretic medications to help control the fluid level in the body, beta blockers to control fast heart rates (atrial fibrillation), device implantation, and, in some cases, heart and/or liver transplantation.

Other treatments such as tafamidis treat the underlying condition by stabilising the amyloid deposits and have been shown to help slow the progression of the condition, help reduce symptoms, increase exercise ability and reduce hospitalisation of people diagnosed with transthyretin amyloidosis when started soon after diagnosis.

Tafamidis works by preventing the formation of amyloid protein through stabilising transthyretin proteins[6]. This decreases the amount of amyloid protein in the body and delays the development of cardiac muscle damage caused by transthyretin amyloidosis.

The estimated prevalent population of transthyretin amyloidosis with cardiomyopathy in the UK in 2023 is 1000, however there is potential for higher numbers due to under diagnosis.

[5] Cardiac amyloidosis | Cardiomyopathy UK
[6] Tafamidis | Drugs | BNF | NICE

According to the Office of National Statistics, the Welsh population makes up 4.7% of the total United Kingdom population[7]. Therefore, it is estimated that there are approximately 50-60 adult patients with transthyretin amyloidosis with cardiomyopathy in Wales.

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).

An EIA was carried out by NICE during the evaluation of tafamidis. For further details, please refer to the NICE website at: https://www.nice.org.uk/guidance/ta984

[7] National population projections - Office for National Statistics

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

Tafamidis is recommended, within its marketing authorisation, as an option for treating wild-type or hereditary transthyretin amyloidosis with cardiomyopathy (ATTR-CM) in adults. Tafamidis is only recommended if 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 or stabilisation 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

National Amyloidosis Centre
University College London
Rowland Hill Street
London
NW3 2PF

2.5 Patient Pathway (Annex i)

Patients are referred to the National Amyloidosis Centre (NAC). Please see Annex i for patient pathway and Annex ii for the standard operating procedure (SOP) for referral to the national centre.

2.6 Mechanism for funding

Tafamidis will only be funded for patients registered via the Blueteq® system and where an appropriately constructed MDT has approved its use within highly specialised centre(s). Where the patient meets the criteria in this policy and the referral is received by an agreed centre(s), 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 tafamidis is prescribed and administered in accordance with its marketing authorisation. Tafamidis, is available as Vyndaqel 61mg capsules. The cost is £10,685.00 for a 30 capsule pack of the 61mg strength (excluding VAT; company's evidence submission). The company has a commercial arrangement. This makes tafamidis 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.

The centre must facilitate informed participation by patient, carer and advocate and to be able to demonstrate this. Provision should be made for patients with communication difficulties.

2.8 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 purchase tafamidis at the agreed discounted price.
- Providers are to ensure the need to approve tafamidis 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. 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)

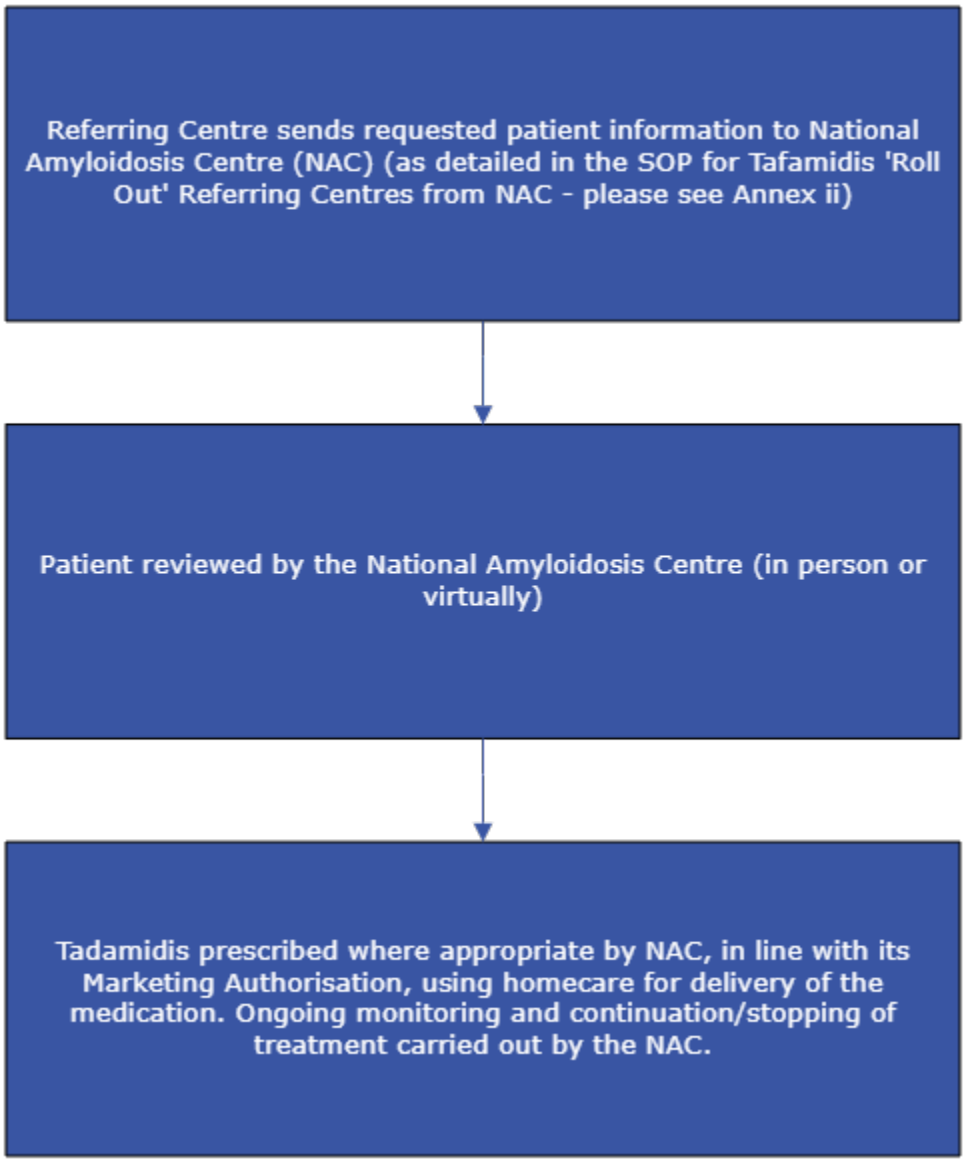
If the patient does not meet the criteria for treatment as outlined in this policy, an Individual Patient Funding Request (IPFR) can be submitted for consideration in line with the All Wales Policy: Making Decisions on Individual Patient Funding Requests. The request will then be considered by the All Wales IPFR Panel.

If 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



Annex ii Standard Operating Procedure for tafamidis 'roll out' for referring centres

For patients who are not intending to attend the National Amyloidosis Centre (NAC);

1. Provision to NAC from Referring Centre of minimum dataset (in one pdf per patient):
   - Patient Contact details (home and mobile tel + address) + NHS number + DoB
   - Referring clinician & GP (with addresses)
   - Latest clinic letter with comorbidities & meds
   - Latest FBC (actual lab results)
   - Latest renal profile (actual lab results)
   - Latest liver profile (actual lab results)
   - Latest NT-proBNP (actual lab results)
   - Latest high sensitivity Troponin T or Troponin I (actual lab results)
   - Serum immunofixation, urine immunofixation (actual lab results) – note: serum electrophoresis alone or urine electrophoresis alone are insufficient (they are not sensitive enough to rule out subtle monoclonal gammopathy)
   - Serum free light chain results (actual lab results)
   - Latest echocardiogram report in full (and CMR report if done)
   - DPD scan report (with Perugini grade) - NAC will arrange transfer of images to NAC
   - TTR genotype (report in full if not done at NAC)
   - Biopsy tissue sent to NAC for amyloid typing (only required if 'biopsy diagnosis')

(If minimum dataset is incomplete, NAC informs referring clinician and waits for complete dataset to be forwarded)

2. NAC clinician has telephone consultation with patient (within 3 weeks) and if eligible, prescribes tafamidis

3. 'Tafamidis pack' sent to patient (with letter from clinician)

4. Letter to referring clinician informing them of commencement of tafamidis (delivered via Healthnet)

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