Medicines Management Programme

Managed Access Protocol – Medicines for the treatment of transthyretin amyloidosis in adult patients with cardiomyopathy

Medicine	Date of addition to Managed Access Protocol
Tafamidis (Vyndaqel®)	01/03/2022
Acoramidis (BEYONTTRA®)	01/12/2025

Approved by	**Prof. Michael Barry, Clinical Lead, MMP**	
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List of abbreviations

ATTR-CM	Transthyretin amyloid cardiomyopathy
BNP	B-type natriuretic peptide
DPD	Diphosphono-1, 2-propanodicarboxylic acid
ECHO	Echocardiography
eGFR	Estimated Glomerular Filtration Rate
HMDP	Hydroxymethylene diphosphonate
HSE	Health Service Executive
HTH	High tech hub
LVEF	Left ventricular ejection fraction
MAP	Managed Access Protocol
MMP	Medicines Management Programme
NYHA	New York Heart Association
NT-proBNP	N-terminal pro b-type natriuretic peptide
PCRS	Primary Care Reimbursement Service
PYP	Pyrophosphate
SmPC	Summary of product characteristics
TTR	Transthyretin

1. Medicines for the treatment of transthyretin amyloidosis in adult patients with cardiomyopathy

There are two medicines referenced in this Managed Access Protocol (MAP) that are available on the High Tech Arrangement for the treatment of wild-type or hereditary/variant transthyretin amyloidosis in adult patients with cardiomyopathy (ATTR-CM).

BEYONTTRA® contains acoramidis. Acoramidis is a specific stabiliser of transthyretin (TTR). Acoramidis was designed to mimic the disease protective genetic variant (T119M), through the formation of hydrogen bonds with adjacent serine residues within both thyroxine binding sites of the tetramer. This interaction enhances the stability of the tetramer, inhibiting its dissociation into monomers, thus slowing the amyloidogenic process that results in ATTR-CM.

One presentation of acoramidis is available on the High Tech Arrangement.

- BEYONTTRA® 356 mg film-coated tablets

Vyndaqel® contains tafamidis. Tafamidis is a selective stabiliser of TTR. Tafamidis binds to TTR at the thyroxine binding sites, stabilising the tetramer and slowing dissociation into monomers, the rate-limiting step in the amyloidogenic process.

One presentation of tafamidis is available on the High Tech Arrangement:

- Vyndaqel® 61 mg capsules

Vyndaqel® 61 mg capsule (tafamidis) corresponds to 80 mg tafamidis meglumine (tafamidis and tafamidis meglumine are not interchangeable on a per mg basis).

1.1 Licensed indication

Acoramidis (BEYONTTRA®) is indicated for the treatment of wild-type or variant transthyretin amyloidosis in adult patients with cardiomyopathy.

Tafamidis (Vyndaqel®) 61 mg capsules are indicated for the treatment of wild-type or hereditary transthyretin amyloidosis in adult patients with cardiomyopathy.

1.2 Reimbursement

Reimbursement of acoramidis or tafamidis on the High Tech Arrangement for the treatment of ATTR-CM is supported only for adult patients who meet the criteria outlined in this MAP. All criteria must be satisfied in order for reimbursement to be supported.

An application for reimbursement approval is required to be submitted on an individual patient basis. The *Medicines for the treatment of transthyretin amyloidosis with cardiomyopathy (ATTR-CM) Application Form* should be completed and sent by secure email to the Health Service Executive (HSE)-Medicines Management Programme (MMP) at mmp@hse.ie.

Table 1 outlines the licensed therapeutic dosages of acoramidis and tafamidis for ATTR-CM. Please refer to the Summary of Product Characteristics (SmPC) for further prescribing information.

Table 1 Licensed therapeutic dosage of acoramidis and tafamidis for the treatment of ATTR-CM in adults

Medicine	Route of administration	Dosage
Acoramidis	Oral	712 mg twice daily
Tafamidis	Oral	61 mg once daily

mg: milligrams

If a patient is recommended and approved for reimbursement of acoramidis or tafamidis, reimbursement is supported in line with the licensed therapeutic dosages specified in Table 1. Reimbursement of dosages in excess of the licensed therapeutic dosages (as outlined in Table 1) is not supported.

Reimbursement is not supported for concomitant use of medicines used in the treatment of ATTR.

See Section 3 for further details on Reimbursement criteria – Requirement for outcome data.

1.3 Reimbursement price

The reimbursement price of the presentations of acoramidis and tafamidis available on the High Tech Arrangement are outlined in Table 2. Commercial-in-confidence arrangements are in place with the marketing authorisation holders to reduce the net acquisition cost of acoramidis and tafamidis to the HSE.

Table 2 Reimbursement codes and prices of the presentations of acoramidis and tafamidis available on the High Tech Arrangement

Strength (pack size)	Code	Reimbursement price*
BEYONTTRA® 356 mg (120 film-coated tablets)	89468	€8,856.00
Vyndaqel® 61mg (30 capsules)	89176	€9,180.00

mg: milligram

*Correct as at 1st December 2025

2. Reimbursement criteria - Initiation

This section outlines the criteria that must be satisfied in order for patients to be recommended for reimbursement of acoramidis or tafamidis for the treatment of ATTR-CM on the High Tech Arrangement.

2.1 Prescribers

Applications for reimbursement approval for acoramidis and tafamidis for the treatment of ATTR-CM on the High Tech Arrangement will only be considered from consultants with specialist registration with the Irish Medical Council in a specialism relevant to the diagnosis and management of ATTR-CM (i.e. cardiology), who have agreed to the terms of this MAP and have been approved by the HSE ('approved consultants').

Approved consultants are responsible for ensuring that the patient or their representative/guardian is aware that the application for reimbursement approval is being made on their behalf.

The prescribing of BEYONTTRA® and Vyndaqel® for approved patients for the treatment of ATTR-CM on the High Tech Arrangement will be confined to the approved consultants and their teams. The governance of the team on the High Tech Hub, including access, rests with the approved consultant.

2.2 Patient age

Applications for reimbursement approval will only be considered for individuals aged ≥ 18 to 90 years at time of application.

2.3 Patient diagnosis

For a positive reimbursement recommendation, approved consultants will be required to confirm a diagnosis of wild-type or variant/hereditary ATTR-CM at the time of application. Approved consultants must provide evidence of a documented diagnosis based upon the following:

1. Exclusion of light chain amyloidosis by serum and urine protein electrophoresis with immunofixation and serum free light chain assay,
2. Diagnosis of amyloidosis confirmed by:
    a) biopsy, where relevant, on cardiac and/or non-cardiac sites, and/or
    b) nuclear scintigraphy (pyrophosphate (PYD), diphosphono-1,2-propanodicarboxylic acid (DPD), hydroxymethylene diphosphonate (HMDP), where relevant, and uptake of Grade 2 or 3,
3. Confirmation by echocardiogram (ECHO), of cardiac end-diastolic interventricular septal wall thickness exceeding 12 mm,
4. History of heart failure, with at least one prior hospitalisation for heart failure or clinical evidence of heart failure (without hospitalisation) manifested in signs or symptoms of volume overload or elevated intracardiac pressures requiring treatment with a diuretic for improvement,
5. Confirmation of TTR genotype (wild-type or variant/hereditary) by genetic testing.

2.3.1 Genetic testing and biopsy

Approved consultants must provide evidence of a documented diagnosis, genetic test and a biopsy report, where relevant.

2.4 Patient's clinical history/status

In line with SmPC's for BEYONTTRA® and Vyndaqel®, applications for reimbursement approval will not be considered for individuals who meet any of the contraindications for treatment as outlined in the relevant SmPCs.

In line with the SmPC's for BEYONTTRA® and Vyndaqel®, and the exclusion criteria from the ATTRibute and ATTR-ACT trials, reimbursement will not be considered in patients:

- With severe heart failure symptoms (defined as New York Heart Association [NYHA] class IV),
- With mild hepatic impairment (defined as liver transaminase levels > twice the upper limit of the normal range),
- With renal failure requiring dialysis and/or an estimated Glomerular Filtration Rate [eGFR] of < 25ml/min/1.73m^2,
- With light chain amyloidosis,
- Using interfering ribonucleic acid drugs or other TTR stabilisers used to treat ATTR-CM.

2.4.1 Heart failure

Approved consultants are required to confirm if there is cardiac involvement associated with the patient's ATTR amyloidosis. The NYHA classification, N-terminal pro b-type natriuretic peptide/ B-type natriuretic peptide (NT-proBNP/BNP) and a recent ECHO are required to be submitted at the time of application.

2.4.2 Hepatic function

Approved consultants are required to confirm hepatic function by submitting a full liver profile at the time of application.

2.4.3 Renal function

Approved consultants are required to confirm renal function by submitting a full renal profile at the time of application.

2.4.4 Exclusion of light chain amyloidosis

Approved consultants are required to confirm that light chain amyloidosis has been excluded at the time of application.

2.5 Patient's medical treatment

Approved consultants are required to provide details of the patient's medical treatment at the time of application.

3. Reimbursement criteria – Requirement for outcome data

Follow-up data may be requested by the MMP for audit purposes and provision of same is a condition of ongoing reimbursement. It is the responsibility of the approved consultant to ensure that the patient or their representative/guardian is aware that the provision of follow-up data is a condition of reimbursement, and that audits may occur during which their personal data will be reviewed.

3.1 Outcome data collection

Patients should be assessed at least every 12 months to determine whether they would benefit from continued treatment with acoramidis (BEYONTTRA®) or tafamidis (Vyndaqel®). An up to date ECHO report and diagnostic testing results may be requested at suitable intervals, and information on whether acoramidis or tafamidis is to be continued or discontinued.

3.2 Discontinuation

Acoramidis (BEYONTTRA®) and Tafamidis (Vyndaqel®) should be discontinued and reimbursement may no longer be supported if the patient progresses to NYHA class IV.

4. Prescribing of acoramidis and tafamidis for approved patients

Please refer to the SmPC's for BEYONTTRA® and Vyndaqel® 61 mg capsules for full prescribing information including monitoring and patient counselling requirements. Only applications from approved consultants will be considered for acoramidis and tafamidis reimbursement.

If a patient is recommended for reimbursement by the HSE-Medicines Management Programme (MMP), the High Tech prescription should be generated on the High Tech Hub (HTH). High Tech prescriptions which are not hub generated for acoramidis or tafamidis will not be eligible for reimbursement by the HSE Primary Care Reimbursement Service (PCRS). Only approved consultants and their teams will have access to generate prescriptions.