Application for individual reimbursement of medicines for the treatment of transthyretin amyloidosis with cardiomyopathy (ATTR-CM)

For MMP Use Only	
Case Reference	*Date Received*

ALL SECTIONS OF THIS FORM MUST BE COMPLETED

Please indicate which treatment this application refers to: Please tick one	
Acoramidis (BEYONTTRA®) ☐ Tafamidis (Vyndaqel®) ☐	
Date of Application:	

Part 1: Patient Details

Name of patient:				
Date of birth:				
Address:				
GMS / DPS / PPS Number: (Please tick and insert number)	GMS	DPS	PPSN	Number:

Part 2: Prescriber Details

Name of Approved Consultant:	
Medical Council Number:	
Contact Details:	Hospital:
	Address:
	Telephone:
	Email:

Please refer to the HSE-Managed Access Protocol for Medicines for the treatment of transthyretin amyloidosis in adult patients with cardiomyopathy when completing part 3 and 4 of this application form

Part 3: Patient Clinical History

Please indicate whether the patient meets the following criteria (*please tick which apply and complete requested detail*):

1. Is the patient aged ≥18-90 years at the time of application? **Yes** ☐ **No** ☐

2. Does the patient have a 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? **Yes** ☐ **No** ☐

Section 1 and section 2 must be completed.

Section 1: Confirmed diagnosis of transthyretin amyloidosis (ATTR) in adult patients with cardiomyopathy, including confirmation of genotype

For a positive recommendation, criteria relating to patient diagnosis must be satisfied. (*Refer to section 2.3 and 2.4 of the managed access protocol*)

3. Has light chain amyloidosis been excluded? **Yes** ☐ **No** ☐

4. Does the patient have a confirmed diagnosis of ATTR amyloidosis with cardiomyopathy (ATTR-CM), defined as either wild-type or hereditary/ variant? **Yes** ☐ **No** ☐

If yes, what is the diagnosis? (please tick one) **Wild-type ATTR** ☐

Hereditary/variant ATTR ☐

5. Please confirm that the diagnosis of ATTR-CM has been established by either (a) and/or (b)[1]:

(a) Presence of amyloid deposits in biopsy tissue (where relevant)? **Yes** ☐ **No** ☐

If yes, please confirm site of biopsy: Cardiac ☐ Non-cardiac ☐

Please attach a biopsy report, where relevant. **Enclosed** ☐

(b) Diagnosis of ATTR-CM by nuclear scintigraphy (PYP/DPD/HMDP[2])? **Yes** ☐ **No** ☐

If yes, what is the uptake? **Grade 2** ☐ **Grade 3** ☐ **Other** ☐

Please attach copy of the nuclear scintigraphy report, where relevant. **Enclosed** ☐

(c) Evidence of cardiac end-diastolic interventricular septal wall thickness >12 mm? **Yes** ☐ **No** ☐

(d) Confirmation of heart failure symptoms defined as New York Heart Association [NYHA] class I, II, III or IV?

Yes, Class I ☐ **Yes, Class II** ☐ **Yes, Class III** ☐ **Yes, Class IV** ☐

Please submit a recent echocardiography report for *all applicants*: **Enclosed** ☐

(e) Confirmation of TTR genotype by genetic testing? **Yes** ☐ **No** ☐

Please attach a copy of the genetic testing investigation for *all applicants*: **Enclosed** ☐

[1] Applications for reimbursement approval will only be considered for patients with a confirmed diagnosis of ATTR amyloidosis, established by biopsy or nuclear scintigraphy or both

[2] PYP: pyrophosphate; DPD: diphosphono-1,2-propanodicarboxylic acid; HMDP: hydroxymethylene diphosphonate

Section 2: Evidence of patient clinical history

For a positive recommendation, evidence relating to patient clinical history must be satisfied (*refer to section 2.4 of the managed access protocol*).

6. Please provide the following information regarding diagnostic testing results obtained at the time of application for <u>all applicants</u>:

		Date recorded	Enclosed
1.	Full renal profile		☐
2.	Full liver profile		☐
3.	BNP/NT-proBNP		☐
4.	Serum free light chains		☐
5.	Immunofixation assay		☐

BNP: B-type natriuretic peptide, NT-proBNP: N-terminal pro b-type natriuretic peptide

Part 4: Patient Medication History

Please confirm the patient's medical treatment at the time of application.

Please provide details:

Medicine	Strength	Dose	Indication

7. Is the patient currently in receipt of any other interfering ribonucleic acid drugs or other TTR stabilisers (including medicines through an early access scheme)?

Yes ☐ **No** ☐

***If yes*,** please provide detail:

Additional space for supporting information

Completed forms should be returned to:

Scan the completed form and return via a secure email (e.g. HSE email or healthmail) to: mmp@hse.ie

Authorisation of Request

Signature of **Approved Consultant**	
Institution	

Data Protection Notice
• The information on this form will be used by the Health Service Executive (HSE) to assess the suitability of the items listed to be provided under Section 20 of the Health (Pricing and Supply of Medical Goods) Act 2013. • Details of prescription items dispensed to the named person may be notified to the HSE by the dispensing pharmacist to ensure that the named person receives the items required. • The named person may access information relating to themselves only, on prescription claims processed in their name by the HSE. • We may share information with the Department of Health, healthcare practitioners and other healthcare bodies. • We may also disclose information to other parties if the law requires us to do so. • The PCRS privacy statement can be located at www.pcrs.ie.