Application for individual reimbursement of patisiran (Onpattro®), vutrisiran (Amvuttra®) or inotersen (Tegsedi®)

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					
Patisiran (Onpattro®) ☐		Vutrisiran (Amvuttra®) ☐		Inotersen (Tegsedi®) ☐	
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 Consultant:		
Medical Council Number:		
Speciality of Prescribing Consultant:	Neurology ☐ Cardiology ☐ Other (Please specify) _____	
Contact Details:	Hospital:	
	Address:	
	Telephone:	
	Email:	

Please refer to the *HSE-Managed Access Protocol-Medicines used in hereditary transthyretin amyloidosis in adult patients with stage 1 or stage 2 polyneuropathy* 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. Please confirm patient age at the time of application _____

Section 1 and section 2 must be completed.

Section 1: Confirmed diagnosis of hereditary transthyretin (hATTR) amyloidosis with stage 1 or stage 2 polyneuropathy

For a positive recommendation, evidence confirming the patient's diagnosis must be provided (*Refer to section 2.3 of the managed access protocol*)

2. Was the diagnosis of ATTR amyloidosis confirmed on tissue biopsy? **Yes** ☐ **No** ☐

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

3. Does the patient have a confirmed diagnosis of hATTR amyloidosis with a documented transthyretin (TTR) mutation? **Yes** ☐ **No** ☐

If yes, what is the patient's known mutation of the TTR gene?

_____

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

4. Has the patient a confirmed diagnosis of hATTR amyloidosis with stage 1 or 2 polyneuropathy? **Yes** ☐ **No** ☐

If yes, please indicate the patient's stage of polyneuropathy disability using the table below:

PND score	Score description	*FAP stage	Stage description	Please choose one:
0	No impairment	0	No symptoms	☐
I	Sensory disturbances, preserved walking capabilities	1	Unimpaired ambulation, mostly mild sensory and motor neuropathy in the lower limbs	☐
II	Impaired walking capabilities but ability to walk without stick or crutches	2	Assistance with ambulation needed; mostly moderate impairment progression to the lower limbs, upper limbs and trunk	☐
IIIA	Walking only with the help of 1 stick or crutch	2	Assistance with ambulation needed; mostly moderate impairment progression to the lower limbs, upper limbs and trunk	☐
IIIB	Walking only with the help of 2 sticks or crutches	2	Assistance with ambulation needed; mostly moderate impairment progression to the lower limbs, upper limbs and trunk	☐
IV	Confined to a wheelchair or bedridden	3	Wheel-chair bound or bedridden; severe sensory and motor neuropathy of all limbs	☐

FAP: Familial amyloidotic polyneuropathy, PND: Polyneuropathy disability
*hATTR amyloidosis with polyneuropathy was formerly known as FAP

5. What is the indication for treatment? (*please tick one*)

Polyneuropathy ☐

Cardiomyopathy ☐

Both of the above ☐

Section 2: Evidence of patient clinical history

For a positive recommendation, criteria relating to patient clinical history must be satisfied.

(*Refer to section 2.4 of the managed access protocol*)

6. Does the patient have severe heart failure symptoms (defined as New York Heart Association [NYHA] class III or IV)?

Yes, Class III ☐ **Yes, Class IV** ☐ **No** ☐

Please submit an up to date echocardiography report for all applicants at the time of application: **Enclosed** ☐

7. Has the patient had a liver transplant? **Yes** ☐ **No** ☐

8. Is a liver transplant planned for the patient? **Yes** ☐ **No** ☐

9. Please provide the following information regarding diagnostic testing results obtained at the time of application for all applicants:

		Date recorded	Enclosed
1.	Full blood count		☐
2.	Full renal profile		☐
3.	Full liver profile		☐
4.	HbA1c		☐
5.	TSH		☐
6.	Vitamin B12		☐
7.	BNP/NT-proBNP		☐
8.	Immunoglobulins		☐
9.	Serum protein electrophoresis		☐
10.	Urine electrophoresis		☐
11.	Serum free light chains		☐
12.	Immunofixation assay		☐
13.	Nerve conduction studies		☐

BNP: B-type natriuretic peptide, NT-proBNP: N-terminal pro b-type natriuretic peptide, TSH: Thyroid stimulating hormone

Part 4: Patient Medication History

10. Is the patient currently in receipt of any other interfering ribonucleic acid drugs or other TTR stabilisers (including medicines through an early access scheme), or other treatments for hATTR amyloidosis?

Yes ☐ **No** ☐

If yes, please provide detail:

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

Please provide details:

Medicine	Strength	Dose	Indication

Part 5: Dosing Information

For hospital administered medicines (patisiran/vutrisiran), outline proposed location of administration (hospital or site name)

For patisiran applications outline:

- Patient weight
- proposed dose of patisiran

Additional space for supporting information

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.

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 **Consultant**	
Institution	