Medicines for Paroxysmal Nocturnal Haemoglobinuria (PNH)

Application Form - Ravulizumab (Ultomiris®)

For MMP Use Only	
Case Reference:	*Date Received:*

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	
Contact Details	Hospital:
	Address:
	Phone:
	Email:

Please refer to the HSE Managed Access Protocol for ravulizumab (Ultomiris®) for PNH when completing Part 3 of this application form

Part 3: Reimbursement criteria - Initiation

Part 3(a): Patient eligibility

1. The patient is aged 18 years or older at the time of application? **Yes:** ☐ **No:** ☐

2. Please outline the weight of the patient (in kilograms, kg) ☐

3. The patient has a confirmed diagnosis of paroxysmal nocturnal haemoglobinuria (PNH)?
   Yes: ☐ **No:** ☐

4. Please outline the indication for treatment:
   a) PNH with haemolysis with clinical symptom(s) indicative of high disease activity ☐
   b) PNH and clinically stable after having been treated with eculizumab for at least the past six months ☐

5. The patient is approved via the centralised funding process for eculizumab for PNH:
   Yes: ☐ **No:** ☐

If the answer to Q5 is NO, please complete ALL sections of this application form.
If the answer to Q5 is YES, please proceed to part 3(c) and complete the remainder of the application form.

Part 3(b): Patient diagnosis

To enable a positive recommendation, evidence confirming a diagnosis of PNH must be provided as part of the application. Please refer to section 2.3 of the managed access protocol for further detail.

Please provide:

i. A flow cytometry report — **Enclosed** ☐

ii. A full blood count (including absolute neutrophil count, B12, folate, serum ferritin) and a haemolytic blood panel (including haemoglobin, bilirubin, haptoglobin, lactate dehydrogenase, reticulocyte count) — **Enclosed** ☐

iii. A biochemistry profile — **Enclosed** ☐

iv. Bone marrow investigations (aspirate, biopsy) — **Enclosed** ☐

v. Genetic testing confirming a phosphatidylinositol glycan anchor biosynthesis class A (PIGA)-gene mutation (if available) — **Enclosed** ☐

vi. Please indicate which, if any, PNH features are present (Please tick all that apply):

Clinical feature	**Yes**	**No**
Haemolysis		
Haemoglobinuria		
Anaemia		
Severe fatigue or weakness		
Headaches		
Thrombosis		
Dyspnoea		
Recurring infections and/or flu-like symptoms		
Fever due to infection		
Chest pain		
Dysphagia		
Abdominal pain		
Oesophageal spasms		
Pulmonary hypertension		
Other (outline below)		

Outline further detail here:

Part 3(c): Patient clinical status

This section must be completed for ALL patients

1. Does the patient have any contraindications for treatment outlined in the SmPC for Ultomiris®?

Yes: ☐ **No:** ☐

Please provide the following information in relation to thrombosis history.

2. Please outline whether the patient has a history of, or is at risk of thrombosis.

Blood count and haemolytic blood panel

3. Please provide a full recent blood count and a haemolytic blood panel (including haemoglobin, bilirubin, haptoglobin, lactate dehydrogenase, reticulocyte count) **if not already provided earlier in section 3(b).**

Enclosed ☐

Part 3(d): Patient Medication History

1. Please confirm:
    i. the patient's current drug therapy at the time of application
    ii. any relevant previous treatments

Please provide details:

Medicine	Strength	Dose	Indication	Current (c)/ Previous (p)

2. The patient is currently in receipt of Ultomiris® or another complement inhibitor used in the treatment of PNH: **Yes:** ☐ **No:** ☐

    If yes, please provide further details:

_____

_____

_____

_____

Please provide the following information in relation to transfusion history.

3. Please outline the transfusion history of the patient for the previous 12 months:

_____

_____

_____

Additional space for supporting information if required

Completed forms should be returned to:
Scan the completed form and return via 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.