Application for individual reimbursement of Obeticholic Acid (Ocaliva®) for the treatment of Primary Biliary Cholangitis (PBC)

For MMP Use Only	
Case Reference	*Date Received*

Date of Application:	Nominated Community Pharmacy: (Name & address – *leave blank if uncertain)*

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 prescribing consultant:	
Medical Council number:	
Contact Details:	Hospital:
	Address:
	Telephone:
	Email:

Please refer to the HSE-Managed Access Protocol for Obeticholic acid for the treatment of PBC 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. This patient is aged 18 years or older at the time of application. **Yes** ☐ **No** ☐

Section 1 and section 2 must be completed.

Section 1: Confirmed diagnosis of Primary Biliary Cholangitis (PBC)

For a positive recommendation, evidence relating to patient diagnosis must be satisfied.

For reimbursement approval, **two** of the options 1-3 must be satisfied (please tick which apply and complete requested detail below).

Refer to section 2.3 of the Managed Access Protocol - Diagnosis

Option 1: A diagnosis of PBC was supported by a history of elevated serum alkaline phosphatase (ALP) levels for at least six months.

Yes ☐ **No** ☐

***If yes*, please provide the relevant ALP levels:**

Serum ALP levels for diagnosis of PBC			
ALP level 1 (U/L):		**Date of blood test:**	
ALP level 2 (U/L):		***Date of blood test:**	

ALP: Alkaline phosphatase
*The duration between the two blood tests should be ≥ six months

Option 2: A diagnosis of PBC was confirmed by:

	Yes	No
Serological positivity of AMA		
Serological positivity of PBC specific antibodies or by antibodies against the major M2 components		

AMA: Anti-mitochondrial antibody; PBC: Primary Biliary Cholangitis

***If yes,* please attach the relevant laboratory report(s)**

Enclosed ☐

Option 3: A diagnosis of PBC was confirmed on liver biopsy.

Yes ☐ **No** ☐

***If yes,* please attach a liver biopsy report** **Enclosed** ☐

Section 2: Patient clinical history

Please indicate the current status of the patient in relation to the following clinical parameters (please tick which apply and complete requested detail below)

Refer to section 2.4 of the Managed Access Protocol - Patient clinical history

The patient has:	Yes	No
Decompensated cirrhosis (e.g. Child-Pugh Class B or C) or a prior decompensation event		
A history or a presence of other concomitant liver diseases, e.g. hepatitis C virus infection, active hepatitis B infection, PSC, alcoholic liver disease, definite autoimmune liver disease or overlap hepatitis, NASH, Gilbert's Syndrome		
A presence of clinical complications of PBC, e.g. liver transplantation, MELD score ≥ 15, portal hypertension with complications		
A history of alcohol abuse or other substance misuse		
Complete biliary obstruction		
A hypersensitivity to the active substance or any of the excipients of Ocaliva®		

MELD: Model for End-stage Liver Disease; NASH: Non-alcoholic Steatohepatitis; PBC: Primary Biliary Cholangitis, PSC: Primary Sclerosing Cholangitis

If **yes** has been answered in the table above, please provide relevant information in the box provided below:

Part 4: Patient Medication History

Evidence of inadequate response to previously trialled ursodeoxycholic acid (UDCA), or where UDCA is not tolerated or is contraindicated.

For reimbursement approval, **one** of options 1-3 must be satisfied (please tick which apply and complete requested detail below).

Refer to section 2.5 of the managed access protocol - Ursodeoxycholic acid

Option 1: Patient has had a trial with UDCA that has resulted in an inadequate response. ☐

A trial of UDCA is defined as a period of at least 12 months of treatment with UDCA, with at least three months at a dose of 13-15 mg/kg/day.

Please provide details:

<table>
<tr><th colspan="3">UDCA treatment</th></tr>
<tr><td>Dose</td><td colspan="2"></td></tr>
<tr><td>Duration of treatment</td><td colspan="2"></td></tr>
<tr><td rowspan="4">Evidence of inadequate response after a trial of UDCA</td><td colspan="2">Serum ALP level or total bilirubin</td></tr>
<tr><td colspan="2">Please tick one:
Serum ALP level ☐ Result: _____ U/L
Total bilirubin level ☐ Result: _____ µmol/L</td></tr>
<tr><td>Date of blood test:</td><td></td></tr>
<tr><td colspan="2">Please attach a copy of the results of the blood test.
Enclosed ☐</td></tr>
</table>

ALP: Alkaline phosphatase; UDCA: ursodeoxycholic acid

Option 2: Patient did not tolerate UDCA and experienced a clinically significant adverse reaction which led to discontinuation of treatment prior to completion of a trial. ☐

Please provide details:

UDCA treatment	
Dose	
Duration of treatment (include start and stop dates)	
Provide details of the clinically significant adverse reaction which led to discontinuation of UDCA prior to completion of a trial:	
Was the adverse reaction reported to the Health Products Regulatory Authority (HPRA)? Yes ☐ No ☐ If yes, please provide the date the adverse reaction was reported: _____	

Option 3: Patient in whom UDCA is contraindicated. ☐

Please provide details:

Provide details of the contraindication, including supporting evidence:

Part 5: Baseline serum ALP levels and total bilirubin

Evidence of baseline serum ALP levels and total bilirubin prior to commencement of treatment with obeticholic acid (within 30 days of date of application)

Refer to section 2.6 of the managed access protocol – Baseline serum ALP levels and total bilirubin (please complete requested detail below)

Please note that where the patient is currently in receipt of UDCA and classified as an inadequate responder, the baseline serum ALP levels and total bilirubin may be the same as those provided to demonstrate an inadequate response to UDCA, as per part 4 option 1.

Please provide details:

Baseline serum ALP level	
Result:	U/L
***Date of blood test:**	
Please attach a copy of the baseline serum ALP levels **Enclosed** ☐	

ALP: Alkaline phosphatase
*Within 30 days of date of application

Please provide details:

Baseline total bilirubin level	
Result:	µmol/L
***Date of blood test:**	
Please attach a copy of the baseline total bilirubin levels **Enclosed** ☐	

*Within 30 days of date of application

Additional space for supporting information

Completed forms should be returned by:

Email (using secure email, e.g. HSE email or healthmail): mmp@hse.ie

Or

Post: Prof Michael Barry, HSE-Medicines Management Programme, Department of Pharmacology and Therapeutics, Trinity Centre for Health Sciences, St James's Hospital, Dublin 8.

Please note that the MMP will always acknowledge receipt of each application.

Authorisation of Request

Signature of **Prescribing 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 names 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.