ALL SECTIONS OF THIS FORM MUST BE COMPLETED

Application for individual reimbursement of voretigene neparvovec (Luxturna®)

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 Prescribing Consultant:	
Medical Council Number:	
Contact Details:	Hospital:
	Address:
	Telephone:
	Email:

Please refer to the HSE-Managed Access Protocol for voretigene neparvovec (Luxturna®) when completing 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 4 years or older at the time of application? **Yes** ☐ **No** ☐

Section 1, Section 2 and Section 3 must be completed.

Section 1: Confirmed genetic diagnosis of biallelic *RPE65* mutations

For a positive recommendation, evidence relating to patient diagnosis must be satisfied. (*Refer to section 2.2.1 of the managed access protocol*)

2. Does the patient have a confirmed genetic diagnosis of biallelic *RPE65* gene mutations?

Yes ☐ **No** ☐

3. If **Yes,** please confirm the classification of the mutation types

Class III ☐ ☐ **Class IV** ☐ ☐ **Class V** ☐ ☐

Please attach a copy of the genetic test confirming the diagnosis of biallelic RPE65 gene mutations: **Enclosed** ☐

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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*)

Please answer question 4 (a & b) and/or 5 (a & b):

4(a) Does the patient currently have **Visual Acuity worse than** 20/60 in the **right eye**? **Yes** ☐ **No** ☐	
Visual Acuity score:	
Date of test (dd/mm/yyyy):	

4(b) Does the patient currently have **Visual Acuity worse than** 20/60 in the **left eye**? **Yes** ☐ **No** ☐	
Visual Acuity score:	
Date of test (dd/mm/yyyy):	

and/or

5(a) Does the patient currently have **Visual Field** < 20 degrees in the **right eye** in any meridian? **Yes** ☐ **No** ☐	
Visual Field reading:	
Date of test (dd/mm/yyyy):	

5(b) Does the patient currently have **Visual Field** < 20 degrees in the **left eye** in any meridian? **Yes** ☐ **No** ☐	
Visual Field reading:	
Date of test (dd/mm/yyyy):	

Please attach a copy of the appropriate test investigations for each eye: **Enclosed** ☐

For a positive recommendation, evidence of sufficient viable retinal cells as determined by non-invasive means, such as Optical Coherence Tomography (OCT) and/or ophthalmoscopy must be satisfied. (Refer to section 2.4 and 2.5 of the managed access protocol)

6. Does the patient currently have sufficient viable retinal cells in each eye?

Right eye: Yes ☐ **No** ☐

Left eye: Yes ☐ **No** ☐

If **Yes,** please confirm how this diagnosis was made:

a) Area of retina within the posterior pole of > 100 µm thickness shown on OCT? ☐

b) ≥ 3 disc areas of retina without atrophy or pigmentary degeneration within the posterior pole? ☐

a) Remaining visual field within 30º of fixation as measured by III4e isopter or equivalent? ☐

Please attach a copy of the appropriate test investigations for each eye: **Enclosed** ☐

7. Does the patient currently have an ocular or periocular infection? **Yes** ☐ **No** ☐

8. Does the patient currently have active intraocular inflammation? **Yes** ☐ **No** ☐

Section 3: Full field stimulus threshold (FST) Baseline information

For reimbursement approval, a baseline full field stimulus threshold (FST) measurement averaged over both eyes up to 90 days prior to treatment with voretigene neparvovec (Luxturna®), using the approved FST Diagnosys System must be provided with the application. (Refer to section 2.6 of the managed access protocol).*

9. Please provide the FST measurement for the **right eye,** prior to the initiation of treatment with voretigene neparvovec (Luxturna®):	Log10(cd.s/m^2)
Date of test (dd/mm/yyyy):	

10. Please provide the FST measurement for the **left eye,** prior to the initiation of treatment with voretigene neparvovec (Luxturna®):	Log10(cd.s/m^2)
Date of test (dd/mm/yyyy):	

11. Please provide the average FST measurement over **both eyes:**	Log10(cd.s/m^2)

Please attach a copy of the FST measurements <u>for each eye</u> using the approved FST Diagnosys System (Please ensure the date of each test is included): **Enclosed** ☐

*There may be circumstances where a baseline FST measurement averaged over both eyes is not attainable. In such cases, an application may be submitted for consideration, however sufficient clinical justification for not attaining the baseline measurement must be clearly outlined. Alternative measurements should be provided in such cases. This information should be outlined in the section *Additional space for supporting information*.

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Additional space for supporting information

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The following confirmations are required when prescribing voretigene neparvovec (Luxturna®):

- Confirmation that voretigene neparvovec (Luxturna®) is being prescribed for a MMP approved patient in accordance with the MAP established in line with the terms of reimbursement approval given by the HSE.
- Confirmation that the prescriber will provide outcome data as requested and provision of this outcome data within the time frame specified with the request, is a condition of ongoing access to apply for reimbursement support of voretigene neparvovec (Luxturna®).
- Confirmation that the prescriber will assist the HSE/MMP in their conduct of audits* through provision of information as requested, to provide assurance that the product is being prescribed in line with HSE reimbursement approval and the MAP.
- Confirmation that the patient or their representative/guardian is aware that the application for reimbursement approval is being made on behalf of the patient and that audits may occur during which their personal data will be reviewed.

* Follow-up data may be requested by the HSE/MMP for audit purposes and provision of same is a condition of ongoing reimbursement.

Completed forms should be returned to:

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

Or

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 **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 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.