# Application for individual reimbursement of medicines for the treatment of Spinal Muscular Atrophy (SMA)

| *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

Nusinersen (Spinraza®) ☐ Onasemnogene abeparvovec (Zolgensma®) ☐

Risdiplam (Evrysdi®) ☐

| 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 the relevant medicine when completing part 3 and 4 of this application form**

## Part 3: Patient Clinical History

**Please complete all questions below.**

**1.** Please confirm the patient age at the time of application

**Confirmed diagnosis of Spinal Muscular Atrophy (SMA)**

*For a positive recommendation, evidence confirming patient diagnosis must be provided. (Refer to appropriate section of the relevant managed access protocol)*

**2.** Does the patient have a diagnosis of Spinal Muscular Atrophy (SMA)? **Yes** ☐ **No** ☐

(a) If yes, what is the subtype? (e.g. Type 1, Type 2, Type 3, N/A)

(b) Does the patient have a homozygous deletion of the SMN1 gene? **Yes** ☐ **No** ☐

(c) Please outline the number of SMN2 copy numbers present

(d) What is the patient's AAV9 antibody titre? (OA applications only)

*OA: Onasemnogene abeparvovec

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

**3.** Is the patient currently in receipt of **OR** has the patient previously been in receipt of any disease modifying treatments for SMA (including medicines through an early access scheme or clinical trial)? Yes ☐ No ☐

If yes, please specify the treatments. Where relevant, outline the reason(s) and date for discontinuation of initial therapy and/or the requirement for further therapy.

**4. Please answer Q4 if requesting to switch patient from nusinersen to risdiplam:**

Does the patient currently meet the discontinuation criteria for nusinersen? (i.e. requiring ongoing ventilation assistance ≥16 hours per day for 21 days in the absence of infection, two consecutive measurements demonstrating worsening motor function, or poor quality of life due to SMA progression)?

Yes ☐ No ☐

## Part 4: Patient Clinical Presentation

**5.** Please outline the patient's physical presentation of disease (e.g. ability to sit/ walk/ stand/ feed orally, presence of muscle weakness/ fatigue/ dysphagia/ tremor/ nystagmus/ scoliosis, requirements for ventilation etc.)

**6**. Please confirm (tick) the assessment scale(s) that will be used to monitor the patient's progress and provide relevant baseline results:

| Scale[1] | Tick relevant scales | Date | Score |
|---|---|---|---|
| 6MWT | | | |
| BSID-III | | | |
| CHOP-INTEND | | | |
| HFMSE | | | |
| HINE-2 | | | |
| MFM-20 | | | |
| MFM-32 | | | |
| RHS | | | |
| RULM | | | |

* Please note, selected scales and baseline levels provided will form part of outcome data requirements for ongoing reimbursement support

## Additional space for supporting information

[1] 6MWT: 6 minute walk test; BSID-III: Bayley Scales of Infant and Toddler Development, Third Edition; CHOP INTEND: Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders; HFMSE: Hammersmith Functional Motor Scale expanded; HINE-2: Hammersmith Infant Neurological Examination, Section 2; MFM-20: Motor Function Measure; RHS Revised Hammersmith Scale; RULM: Revised Upper Limb Module.

| 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.