Australian Government
Services Australia

medicare

PBS

Spinal muscular atrophy paediatric – nusinersen or risdiplam – initial authority application

When to use this form

Use this form to apply for **initial** PBS-subsidised nusinersen or risdiplam for patients aged 18 years or younger with spinal muscular atrophy (SMA) who are either:

- untreated with gene therapy for this condition
- initiating or returning to nusinersen or risdiplam after treatment with gene therapy for this condition due to a regression in a developmental state.

Important information

Initial applications to start PBS-subsidised treatment must be in writing and must include sufficient information to determine the patient's eligibility according to the PBS criteria.

Under no circumstances will phone approvals be granted for SMA **initial** authority applications.

Where the term 'gene therapy' appears, it refers to onasemnogene abeparvovec, and the term 'disease modifying treatment' refers to nusinersen or risdiplam.

Recognised hospitals in the management of SMA are Queensland Children's Hospital (Brisbane), Royal Children's Hospital Melbourne, Monash Children's Hospital (Melbourne), John Hunter Hospital (Newcastle), Sydney Children's Hospital Randwick, Children's Hospital at Westmead, Adelaide Women and Children's Hospital and Perth Children's Hospital.

The information in this form is correct at the time of publishing and may be subject to change.

Continuing treatment

This form is ONLY for **initial** treatment.

After a written authority application for **initial** treatment has been approved, applications for **continuing** treatment can be made in real time using the **Online PBS Authorities** system or by phone.
Call 1800 700 270 Monday to Friday, 8 am to 5 pm, local time.

Call charges may apply.

Section 100 arrangements for nusinersen and risdiplam

These items are available to a patient who is attending:

- an approved private hospital, **or**
- a public hospital

and is a:

- day admitted patient
- non-admitted patient, **or**
- patient on discharge.

These items are not available as a PBS benefit for in-patients of a public hospital.

The hospital name and provider number must be included in this authority form.

Treatment specifics

Patients receiving treatment with nusinersen **must not exceed 4 loading doses** (at days 0, 14, 28, 63) under this restriction.

Patients receiving treatment with risdiplam must have the quantity of drug prescribed in accordance with the recommended dosing in the approved Product Information and **must not exceed 3 units**.

For more information

Go to **servicesaustralia.gov.au/healthprofessionals**

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Patient's details

1 Medicare card number

Ref no.

or

Department of Veterans' Affairs card number

2 Mr ☐ Miss ☐ Other

Family name

First given name

3 Date of birth (DD MM YYYY)

Prescriber's details

4 Prescriber number

5 Dr ☐ Mr ☐ Mrs ☐ Miss ☐ Ms ☐ Other

Family name

First given name

6 Business phone number (including area code)

Alternative phone number (including area code)

Hospital details

7 Hospital name

This hospital is a:

☐ public hospital

☐ private hospital

8 Hospital provider number

Conditions and criteria

To qualify for PBS authority approval, the following conditions must be met.

9 Is the patient, aged 18 years or younger, being treated by, or in consultation with, a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic?

No ☐

Yes ☐ for risdiplam patients with type I/II/IIIa SMA

▶ ***Go to 11***

Yes ☐ for risdiplam patients with pre-symptomatic SMA and all nusinersen patients where the neuromuscular clinic is part of a recognised hospital in the management of SMA

▶ ***Go to 11***

Not applicable ☐ for risdiplam patients with IIIb/IIIc SMA

▶ ***Go to 10***

10 The patient, aged 18 years or younger, is being treated by:

☐ a specialist medical practitioner experienced in the diagnosis and management of SMA

or

☐ a medical practitioner directed to prescribe this benefit by a specialist medical practitioner experienced in the diagnosis and management of SMA.

11 Will the treatment be given concomitantly with best supportive care for this condition?

No ☐

Yes ☐

12 Is the patient untreated with gene therapy?

No ☐ ▶ ***Go to 26***

Yes ☐ ▶ ***Go to 13***

Not applicable ☐ as this is for IIIb/IIIc SMA ▶ ***Go to 13***

13 This condition has genetic confirmation of:

☐ 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene

or

☐ deletion of one copy of the SMN1 gene, in addition to a pathogenic/likely pathogenic variant in the remaining single copy of the SMN1 gene

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For pre-symptomatic SMA	▶ *Go to 14*
For symptomatic type I, II or IIIa SMA:	
• risdiplam	▶ *Go to 20*
• nusinersen	▶ *Go to 22*
For symptomatic type IIIb/IIIc SMA	▶ *Go to 17*

14 Does the patient have pre-symptomatic SMA and is under 36 months of age prior to commencing treatment?

No ☐
Yes ☐

15 The patient has genetic confirmation, as determined by quantitative polymerase chain reaction (qPCR) or multiple ligation dependent probe amplification (MLPA), that there are:

☐ 1 to 2 copies of the survival motor neuron 2 (SMN2) gene

or

☐ 3 copies of the SMN2 gene (applicable to nusinersen ONLY).

16 Is a copy of the results substantiating the number of SMN2 gene copies determined by qPCR or MLPA included with this application?

No ☐ ▶ ***Ineligible***
Yes ☐ ▶ ***Go to 32***

17 Is the patient's medical history consistent with a diagnosis of type IIIb/IIIc SMA?

No ☐
Yes ☐

18 The patient:

☐ is initiating PBS-subsidised treatment for untreated disease

or

☐ has initiated treatment via non-PBS supply

19 Is this benefit the sole PBS-subsidised disease modifying treatment?

No ☐
Yes ☐ ▶ ***Go to 21***

20 Is this treatment in combination with PBS-subsidised nusinersen for this condition?

No ☐
Yes ☐

21 Will PBS-subsidised treatment with this drug be ceased when invasive permanent assisted ventilation is required in the absence of a potentially reversible cause?

No ☐
Yes ☐ ▶ ***Go to 23***

22 Is this treatment in combination with PBS-subsidised risdiplam for this condition?

No ☐
Yes ☐

23 Indicate the patient's SMA type and defined signs **and** symptoms that the patient has experienced:

☐ Type I SMA with an onset before 6 months of age **and** at least one of the following:

- ☐ failure to meet or regression in ability to perform age-appropriate motor milestones
- ☐ proximal weakness
- ☐ hypotonia
- ☐ absence of deep tendon reflexes
- ☐ failure to gain weight appropriate for age
- ☐ any active chronic neurogenic changes
- ☐ a compound muscle action potential below normative values for an age-matched child

▶ ***Go to 24***

or

☐ Type II SMA with an onset between 6 and 18 months of age **and** at least one of the following:

- ☐ failure to meet or regression in ability to perform age-appropriate motor milestones
- ☐ proximal weakness
- ☐ weakness in trunk righting/derotation
- ☐ hypotonia
- ☐ absence of deep tendon reflexes
- ☐ failure to gain weight appropriate for age
- ☐ any active chronic neurogenic changes
- ☐ a compound muscle action potential below normative values for an age-matched child

▶ ***Go to 24***

or

☐ Type IIIa SMA with an onset between 18 months and 36 months of age **and** at least one of the following:

- ☐ failure to meet or regression in ability to perform age-appropriate motor milestones
- ☐ proximal weakness
- ☐ hypotonia
- ☐ absence of deep tendon reflexes
- ☐ failure to gain weight appropriate for age
- ☐ any active chronic neurogenic changes
- ☐ a compound muscle action potential below normative values for an age-matched child

▶ ***Go to 24***

or

☐ Type IIIb/IIIc SMA with an onset from 3 years but before 19 years of age **and** at least one of the following:

- ☐ failure to meet or regression in ability to perform age-appropriate motor milestones
- ☐ proximal weakness
- ☐ hypotonia
- ☐ absence of deep tendon reflexes
- ☐ any active denervation or chronic neurogenic changes found on electromyography
- ☐ a compound muscle action potential below normative values for an age-matched child

▶ ***Go to 25***

24 Provide the patient's age (in months) at the onset of these signs/symptoms

months ▶ ***Go to 32***

25 Provide the patient's age (in years) at the onset of these signs/symptoms

years ▶ ***Go to 32***

For a patient with type I or pre-symptomatic SMA initiating or returning after gene therapy

26 The patient has:

☐ had gene therapy as the most recent PBS authority approval for this condition

and

☐ experienced a regression in a developmental state (refer to **Definitions** on the next page) that is:

- ☐ apparent for at least 3 months

and

- ☐ not due to an acute concomitant illness

and

- ☐ not due to non-compliance to best supportive care

and

- ☐ verified by another clinician in the treatment team.

27 Provide details of the regression and the verifying clinician

Refer to **Definitions** on the next page for the childhood developmental states (1-9).

Full name of verifying clinician

Profession of verifying clinician (for example, medical practitioner, nurse, physiotherapist)

Patient's overall or best achieved development state (1-9)

Patient's current overall development state (0-8, this value must be lower than the value provided above)

28 The patient is initiating or returning to:

☐ risdiplam to treat symptomatic Type 1 SMA ▶ ***Go to 29***

or

☐ nusinersen to treat symptomatic Type 1 SMA ▶ ***Go to 30***

or

☐ nusinersen to treat pre-symptomatic SMA ▶ ***Go to 30***

29 Is this treatment in combination with PBS-subsidised nusinersen for this condition?

No ☐ ▶ ***Go to 31***

Yes ☐

30 Is this treatment in combination with PBS-subsidised risdiplam for this condition?

No ☐

Yes ☐

31 Has the condition progressed to a point where invasive permanent assisted ventilation is required in the absence of a potentially reversible cause (that is, ventilation via tracheostomy for at least 16 hours per day)?

No ☐

Yes ☐

32 Indicate the number of units prescribed in accordance with the recommended dosing in the approved Production Information (for **risdiplam** application only)

unit(s)

Checklist

33 The relevant attachments need to be provided with this form.

☐ The completed authority prescription form(s).

☐ A copy of the results substantiating the number of SMN2 gene copies determined by qPCR or MLPA (if you answered Yes at **question 16**).

Privacy notice

34 Personal information is protected by law (including the *Privacy Act 1988*) and is collected by Services Australia for the purposes of assessing and processing this authority application.

Personal information may be used by Services Australia, or given to other parties where the individual has agreed to this, or where it is required or authorised by law (including for the purpose of research or conducting investigations).

More information about the way in which Services Australia manages personal information, including our privacy policy, can be found at **servicesaustralia.gov.au/privacypolicy**

Prescriber's declaration

You do not need to **sign** the declaration if you complete this form using Adobe Acrobat Reader and return this form through Health Professional Online Services (HPOS) at **servicesaustralia.gov.au/hpos**

35 I declare that:

- I am aware that this patient must meet the criteria listed in the current Schedule of Pharmaceutical Benefits to be eligible for this medicine.
- I have informed the patient that their personal information (including health information) will be disclosed to Services Australia for the purposes of assessing and processing this authority application.
- I have provided the completed authority prescription form(s) and the relevant attachments as specified in the Pharmaceutical Benefits Scheme restriction.
- the information I have provided in this form is complete and correct.

I understand that:

- giving false or misleading information is a serious offence.

☐ I have read, understood and agree to the above.

Date (DD MM YYYY) (you **must** date this declaration)

Prescriber's signature (**only** required if returning by post)

If returning by post, print and sign by hand.

Returning this form

Return this form, the authority prescription form(s) and any relevant attachments:

- **online** (no signature required), upload through HPOS at **servicesaustralia.gov.au/hpos**

  or

- by post (signature required) to

  Services Australia
  Complex Drugs Programs
  Reply Paid 9826
  HOBART TAS 7001

Reset form

Definitions

Various childhood developmental states (1 to 9) are listed below, some followed by further observations (a up to d). Where at least one developmental state or observation is no longer present, that developmental state has regressed.

0 Absence of developmental states (1 to 9) listed below:

1 Rolls from side to side on back

2 Child holds head erect for at least 3 seconds unsupported

3 Sitting, but with assistance

4 Sitting without assistance:

- **(a)** Child sits up straight with the head erect for at least 10 seconds
- **(b)** Child does not use arms or hands to balance body or support position.

5 Hands and knees crawling:

- **(a)** Child alternately moves forward or backwards on hands and knees
- **(b)** The stomach does not touch the supporting surface
- **(c)** There are continuous and consecutive movements at least 3 in a row.

6 Standing with assistance:

- **(a)** Child stands in upright position on both feet, holding onto a stable object (for example, furniture) with both hands and without leaning on object
- **(b)** The body does not touch the stable object, and the legs support most of the body weight
- **(c)** Child thus stands with assistance for at least 10 seconds.

7 Standing alone:

- **(a)** Child stands in upright position on both feet (not on the toes) with the back straight
- **(b)** The leg supports 100% of the child's weight
- **(c)** There is no contact with a person or object
- **(d)** Child stands alone for at least 10 seconds.

8 Walking with assistance:

- **(a)** Child is in an upright position with the back straight
- **(b)** Child makes sideways or forced steps by holding onto a stable object (for example, furniture) with 1 or both hands
- **(c)** One leg moves forward while the other supports part of the body weight
- **(d)** Child takes at least 5 steps in this manner.

9 Walking alone:

- **(a)** Child takes at least 5 steps independently in upright position with the back straight
- **(b)** One leg moves forward while the other supports most of the body weight
- **(c)** There is no contact with a person or object.