

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

## Online PBS Authorities



You do not need to complete this form if you use the **Online PBS Authorities** system.

For more information and how to access the **Online PBS Authorities** system, go to [servicesaustralia.gov.au/hppbsauthorities](https://servicesaustralia.gov.au/hppbsauthorities)

## When to use this form

Use this form to apply for **initial** PBS-subsidised nusinersen or risdiplam for patients 18 years or under 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 can be made using the **Online PBS Authorities** system or 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 an 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.

## 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](https://servicesaustralia.gov.au/healthprofessionals)



**10** Is the patient, under 36 months of age prior to commencing treatment, being treated by, or in consultation with, a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic of a recognised hospital in the management of SMA?

Yes  **Go to 12**

No

**11** The patient, under 36 months of age prior to commencing treatment, is being treated by:

a specialist medical practitioner experienced in the diagnosis/management of SMA

**or**

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

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

Yes

No

**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

**14** 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

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

Yes  **Go to 40**

No  **Ineligible**

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

Yes

No

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

Yes

No  **Go to 25**

**18** Is the patient, 18 years or under, being treated by, or in consultation with, a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic of a recognised hospital in the management of SMA?

Yes

No

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

Yes

No  **Go to 26**

**20** Is the patient, 18 years or under, being treated by, or in consultation with, a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic of a recognised hospital in the management of SMA?

Yes  **Go to 22**

No

**21** The patient, 18 years or under, is being treated by a:

specialist medical practitioner experienced in the diagnosis/management of SMA

**or**

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

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

Yes

No

**23** The patient:

is initiating PBS-subsidised treatment for untreated disease

**or**

has initiated treatment via non-PBS supply

**24** Is this the sole PBS-subsidised disease modifying treatment?

Yes

No

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

Yes

No

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

Yes

No

**27** 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

**28** Indicate the patient's SMA type, and the 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 29**

**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 29**

**or**

- Type IIIa SMA with an onset between 18 months and 36 months (3 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
  - 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 29**

**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 30**

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

months

▶ **Go to 40**

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

years

▶ **Go to 40**

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

**31** Is the patient, 18 years or under, being treated by, or in consultation with, a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic of a recognised hospital in the management of SMA?

Yes

No

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

Yes

No  ▶ **Go to 35**

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

Yes

No

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

Yes

No

**35** The patient has had gene therapy as the most recent PBS authority approval for:

symptomatic type I SMA

**or**

pre-symptomatic SMA (not applicable for **risdiplam** application)

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

Yes

No

**37** 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)?

Yes

No

**38** The patient has experienced a regression in a developmental state (refer to **Definitions** on page 6 of this form) 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.

**39** Provide details of the regression and the verifying clinician

Refer to **Definitions** on page 6 of this form for the childhood developmental states (1-9).

Full name of the verifying clinician

Profession of the 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)

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

unit(s)

## Checklist

**41**  The relevant attachments need to be provided with this form.

Details of the proposed prescription(s).

A copy of the results substantiating the number of SMN2 gene copies determined by qPCR or MLPA (if you answered Yes at **question 15**).

## Privacy notice

**42** 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](https://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](https://servicesaustralia.gov.au/hpos)

**43** 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 details of the proposed prescription(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)

## Returning this form

Return this form, details of the proposed prescription(s) and any relevant attachments:

- **online** (no signature required), upload through HPOS at [servicesaustralia.gov.au/hpos](https://servicesaustralia.gov.au/hpos)  
**or**
- by post (signature required) to  
Services Australia  
Complex Drugs Programs  
Reply Paid 9826  
HOBART TAS 7001

## Definitions

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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.