Australian Government
Services Australia

medicare

PBS

Spinal muscular atrophy – onasemnogene abeparvovec – 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**

When to use this form

Use this form to apply for PBS-subsidised onasemnogene abeparvovec for patients weighing up to 21 kg with spinal muscular atrophy (SMA) who are either:

- untreated with disease modifying therapies for this condition
- switching from a disease modifying therapy to onasemnogene abeparvovec for this condition.

Important information

Authority applications for onasemnogene abeparvovec 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 authority applications for onasemnogene abeparvovec.

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.

Accredited treatment centres and suppliers are those organisations accredited by the Gene Technology Regulator under section 92 of the *Gene Technology Act 2000*. The following website provides a list of accredited organisations and may update without notice:
ogtr.gov.au/what-weve-approved/accredited-organisations

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

State the weight of the patient in kilograms and request the appropriate product pack presentation with respect to the mix of 5.5 mL and 8.3 mL vials.

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

Section 100 arrangements

This item is available to a patient who is attending a:

- public participating hospital recognised in managing SMA, **or**
- public hospital recognised in managing SMA

and is a:

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

This item is 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 must be undergoing treatment with onasemnogene abeparvovec once ONLY in a lifetime.

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 Family name

First given name

3 Date of birth (DD MM YYYY)

4 Patient's current weight

kg

Prescriber's details

5 Prescriber number

6 Family name

First given name

7 Business phone number (including area code)

Alternative phone number (including area code)

Hospital details

8 Hospital name

and

☐ this hospital is a public hospital

9 Hospital provider number

Conditions and criteria

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

10 The patient, weighing 21 kg or less, has:

☐ symptomatic Type I, II or IIIa SMA

or

☐ pre-symptomatic SMA with 1 to 3 copies of the survival motor neuron 2 (SMN2) gene, where a disease modifying therapy was initiated prior to 36 months of age

or

☐ pre-symptomatic SMA with 1 to 3 copies of the SMN2 gene, who is no older than 36 months of age and untreated with disease modifying therapies

11 Is the patient 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 ☐

12 The patient is being treated in a treatment centre that:

☐ is recognised in the management of SMA

and

☐ is accredited in the use of this gene technology by the relevant authority

and

☐ has sourced or will source this product from an accredited supplier, as specified in the administrative notes to this listing.

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

Yes ☐

No ☐

MCA0PB330 2611

14 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 ☐

15 Has the patient had prior treatment with nusinersen and/or risdiplam for this condition?

Yes ☐ and PBS-subsidised treatment with the replaced disease modifying agent has ceased or will cease prior to initiating this gene therapy ▶ ***Go to 20***

No ☐

16 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.

17 The patient has:

☐ symptomatic Type 1 SMA with an onset before 6 months of age and experienced at least one of the following defined signs and symptoms:

- ☐ 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 18***

or

☐ symptomatic Type 2 SMA with an onset between 6 months and 18 months of age and experienced at least one of the following defined signs and symptoms:

- ☐ failure to meet or regression in ability to perform age-appropriate motor milestones
- ☐ proximal weakness
- ☐ weakness in truck 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 18***

or

☐ symptomatic Type 3 SMA with an onset between 18 months and 36 months of age and experienced at least one of the following defined signs and symptoms:

- ☐ 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 18***

or

☐ pre-symptomatic SMA

and

- ☐ has genetic confirmation that there are **1** to **2** copies of the SMN2 gene determined by quantitative polymerase chain reaction (qPCR) or multiple ligation dependent probe amplification (MLPA)

or

- ☐ has genetic confirmation that there are **3** copies of the SMN2 gene determined by qPCR or MLPA.

▶ ***Go to 19***

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

months

19 Provide details of the genetic testing

Date of pathology report (DD MM YYYY)

Name of pathology provider

Unique identifying serial number/code that links the genetic test result to the patient

Checklist

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

☐ Details of the proposed prescription(s) stating the correct combination of 5.5 mL and 8.3 mL vials based on the patient's weight.

Privacy notice

21 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**

22 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)

If returning by post, print and sign by hand.

Reset form

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**
- by post (signature required) to

  Services Australia
  Complex Drugs Programs
  Reply Paid 9826
  HOBART TAS 7001