



PB 20 of 2026

National Health (Highly Specialised Drugs Program) Special Arrangement Amendment (March Update) Instrument 2026

National Health Act 1953

I, REBECCA RICHARDSON, Assistant Secretary, PBS Listing, Pricing and Policy Branch, Technology Assessment and Access Division, Department of Health, Disability and Ageing, delegate of the Minister for Health and Ageing, make this Instrument under subsection 100(2) of the *National Health Act 1953*.

Dated 26 February 2026

REBECCA RICHARDSON
Assistant Secretary
PBS Listing, Pricing and Policy Branch
Technology Assessment and Access Division

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1. Name

- (1) This instrument is the *National Health (Highly Specialised Drugs Program) Special Arrangement Amendment (March Update) Instrument 2026*.
- (2) This Instrument may also be cited as PB 20 of 2026.

2. Commencement

- (1) Each provision of this instrument specified in column 1 of the table commences, or is taken to have commenced, in accordance with column 2 of the table. Any other statement in column 2 has effect according to its terms.

Commencement information		
Column 1	Column 2	Column 3
Provisions	Commencement	Date/Details
1. The whole of this instrument	1 March 2026	1 March 2026

Note: This table relates only to the provisions of this instrument as originally made. It will not be amended to deal with any later amendments of this instrument.

- (2) Any information in column 3 of the table is not part of this instrument. Information may be inserted in this column, or information in it may be edited, in any published version of this instrument.

3. Authority

This instrument is made under subsection 100(2) of the *National Health Act 1953*.

4. Schedules

Each instrument that is specified in a Schedule to this instrument is amended or repealed as set out in the applicable items in the Schedule concerned, and any other item in a Schedule to this instrument has effect according to its terms.

Schedule 1—Amendments

National Health (Highly Specialised Drugs Program) Special Arrangement 2021 (PB 27 of 2021)

[1] Schedule 1, entry for Etanercept in the form Injections 50 mg in 1 mL single use pre-filled syringes, 4

insert in the columns in the order indicated and in alphabetical order for the column headed “Brand”:

	Nepexto	C12120 C17706 C17707 C17713 C17724 C17725	See Schedule 2	See Schedule 2
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[2] Schedule 1, entry for Nusinersen

(a) *omit from the column headed “Circumstances”:* C12672 C12676 C13222 C13270 C14370 C14421

(b) *omit from the column headed “Circumstances”:* C15066

(c) *omit from the column headed “Circumstances”:* C15116

(d) *insert in numerical order in the column headed “Circumstances”:* C18053 C18073 C18076 C18077 C18078 C18094 C18101 C18110

[3] Schedule 1, entry for Ravulizumab in each of the forms: Solution concentrate for I.V. infusion 300 mg in 3 mL; and Solution concentrate for I.V. infusion 1,100 mg in 11 mL

insert in numerical order in the column headed “Circumstances”: C18042 C18043 C18044 C18045 C18046 C18057 C18098 C18103

[4] Schedule 1, entry for Risdiplam

(a) *omit from the column headed “Circumstances”:* C14372

(b) *omit from the column headed “Circumstances”:* C15990 C16257

(c) *insert in numerical order in the column headed “Circumstances”:* C18087 C18090 C18118

[5] Schedule 1, entry for Tenofovir with emtricitabine and efavirenz

insert as first entry:

Tablet containing tenofovir disoproxil fumarate 300 mg with emtricitabine 200 mg and efavirenz 600 mg (s19A)	Oral	Efavirenz, Emtricitabine and Tenofovir Disoproxil Fumarate Tablet 600 mg/200 mg/300 mg (Camber, USA)	C4470 C4522	60	5
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[6] Schedule 2, entry for Nusinersen [Maximum quantity: 1 dose; Maximum repeats: 3]

omit from the column headed "Circumstances": C12672 C12676 C13222 C13270 C14370 C14421 C15066 C15116 C18078 C18094 C18101 C18110 substitute: C18053 C18073 C18076 C18077

[7] Schedule 2, entry for Ravulizumab

substitute:

Ravulizumab	C14744 C14780 C14791 C14797 C17055 C17605 C17606 C17610 C17630 C18044 C18046 C18103	1 dose	0
	C18045	1 dose	1
	C14747 C14748 C14749 C16368 C16398 C17609 C17629 C17631 C17761 C18042 C18043 C18057 C18098	1 dose	2

[8] Schedule 2, entry for Risdiplam [Maximum quantity: 1; Maximum repeats: 0]

- (a) omit from the column headed "Circumstances": C14372
- (b) omit from the column headed "Circumstances": C15990 C16257
- (c) insert in numerical order in the column headed "Circumstances": C18087 C18090 C18118

[9] Schedule 3, entry for Nusinersen

(a) omit:

Nusinersen	C12672	Symptomatic Type I, II or IIIa spinal muscular atrophy (SMA) Initial treatment - Loading doses Must be treated by 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; 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. The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR The condition must have genetic confirmation of 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; AND Patient must have experienced at least two of the defined signs and symptoms of SMA type I, II or IIIa prior to 3 years of age; AND The treatment must not be in combination with PBS-subsidised treatment with risdiplam for this condition; AND The treatment must be given concomitantly with best supportive care for this condition; AND	Compliance with Written Authority Required procedures
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C12676	<p>The treatment must not exceed four loading doses (at days 0, 14, 28 and 63) under this restriction; AND Patient must be untreated with gene therapy. Patient must be 18 years of age or under. Defined signs and symptoms of type I SMA are: i) Onset before 6 months of age; and ii) Failure to meet or regression in ability to perform age-appropriate motor milestones; or iii) Proximal weakness; or iv) Hypotonia; or v) Absence of deep tendon reflexes; or vi) Failure to gain weight appropriate for age; or vii) Any active chronic neurogenic changes; or viii) A compound muscle action potential below normative values for an age-matched child. Defined signs and symptoms of type II SMA are: i) Onset between 6 and 18 months; and ii) Failure to meet or regression in ability to perform age-appropriate motor milestones; or iii) Proximal weakness; or iv) Weakness in trunk righting/derotation; or v) Hypotonia; or vi) Absence of deep tendon reflexes; or vii) Failure to gain weight appropriate for age; or viii) Any active chronic neurogenic changes; or ix) A compound muscle action potential below normative values for an age-matched child. Defined signs and symptoms of type IIIa SMA are: i) Onset between 18 months and 3 years of age; and ii) Failure to meet or regression in ability to perform age-appropriate motor milestones; or iii) Proximal weakness; or iv) Hypotonia; or v) Absence of deep tendon reflexes; or vi) Failure to gain weight appropriate for age; or vii) Any active chronic neurogenic changes; or viii) A compound muscle action potential below normative values for an age-matched child. Application for authorisation of initial treatment must be in writing and must include: (a) a completed authority prescription form; and (b) a completed Spinal muscular atrophy PBS Authority Application Form which includes the following: i) specification of SMA type (I, II or IIIa); and (ii) sign(s) and symptom(s) that the patient has experienced; and (iii) patient's age at the onset of sign(s) and symptom(s).</p>	Compliance with Written Authority Required procedures
	<p>Spinal muscular atrophy (SMA) Initial treatment occurring after onasemnogene abeparvovec therapy in a patient with one of: (i) Type 1 SMA, or, (ii) pre-symptomatic SMA Patient must have experienced a regression in a developmental state listed below (see 'Definition') despite treatment with gene therapy - confirm that this: (i) not due to an acute</p>	

concomitant illness; (ii) not due to non-compliance to best-supportive care, (iii) apparent for at least 3 months, (iv) verified by another clinician in the treatment team - state the full name of this clinician plus their profession (e.g. medical practitioner, nurse, physiotherapist; this is not an exhaustive list of examples); AND

The treatment must not be a PBS-subsidised benefit where the condition has progressed to a point where invasive permanent assisted ventilation (i.e. ventilation via tracheostomy tube for at least 16 hours per day) is required in the absence of potentially reversible causes; AND

The treatment must be given concomitantly with best supportive care for this condition; AND
The treatment must not be in combination with PBS-subsidised treatment with risdiplam for this condition.

Must be treated by 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; 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; AND

Patient must be undergoing treatment under this Treatment phase listing once only - for continuing treatment beyond this authority application, refer to the drug's relevant 'Continuing treatment' listing for the patient's SMA type.

Patient must have a prior authority approval for any drug PBS-listed for symptomatic Type 1 SMA, with at least one approval having been for gene therapy; OR

Patient must have a prior authority approval for any drug PBS-listed for pre-symptomatic SMA, with at least one approval having been for gene therapy.

The authority application must be made in writing and must include:

- (1) a completed authority prescription form; and
- (2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).

Do not resubmit previously submitted documentation concerning the diagnosis and type of SMA.

Confirm that a previous PBS authority application has been approved for one of the following:

- (i) Symptomatic Type 1 SMA; or
- (ii) Pre-symptomatic SMA treated with nusinersen.

Definition:

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/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 (e.g. 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 (e.g. 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.
- Confirm which developmental state has regressed by: (i) stating the overall developmental state (1 - 9) the patient was in at the time of gene therapy, or, the best developmental state achieved since gene therapy, and (ii) stating the patient's current overall developmental state (i.e. a number that is lower than stated in (i)).
- Where the patient has neither regressed from a developmental state nor reached the next developmental state, PBS-subsidy of this benefit is not available.

C13222

Symptomatic type IIIB/IIIC spinal muscular atrophy (SMA)
 Initial PBS-subsidised treatment in a child
 The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR
 The condition must have genetic confirmation of 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;
 AND
 Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug.
 Patient must be of an age that is prior to their 19th birthday at the time of this authority application; AND
 Patient must have SMA type III where the onset of signs/symptoms of SMA first occurred after their 3rd birthday, but before their 19th birthday (SMA type IIIB/IIIC).
 Must be treated by 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; 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; AND

Compliance with Written Authority Required procedures

C13270	<p>Patient must be undergoing initial PBS-subsidised treatment for untreated disease - prescribe up to 3 repeat prescriptions to enable dosing occurring at days: 0 (original prescription), 14 (repeat 1), 28 (repeat 2), 63 (repeat 3) (i.e. the loading doses); OR</p> <p>Patient must be undergoing initial PBS-subsidised treatment, but the patient has initiated treatment via non-PBS supply (e.g. clinical trial, sponsor compassionate access) - prescribe zero repeat prescriptions where loading doses are complete; AND</p> <p>Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment.</p> <p>The authority application must be made in writing and must include:</p> <p>(1) a completed authority prescription form; and</p> <p>(2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).</p> <p>Signs and symptoms of spinal muscular atrophy in the context of this PBS restriction are:</p> <p>(i) Failure to meet or regression in ability to perform age-appropriate motor milestones,</p> <p>(ii) Proximal weakness,</p> <p>(iii) Hypotonia,</p> <p>(iv) Absence of deep tendon reflexes,</p> <p>(v) Any active denervation or chronic neurogenic changes found on electromyography,</p> <p>(vi) A compound muscle action potential below normative values for an age-matched child.</p> <p>In this authority application, confirm:</p> <p>(1) the patient's medical history is consistent with a diagnosis of type IIIB/IIIC spinal muscular atrophy,</p> <p>(2) which of the above (i to vi) (at least 1) were present after their 3rd birthday, but before their 19th birthday,</p> <p>(3) the age of the patient (rounded to the nearest year) when the first sign/symptom was observed.</p>	Compliance with Written Authority Required procedures
	<p>Spinal muscular atrophy (SMA)</p> <p>Initial PBS-subsidised treatment in an adult who did not initiate PBS subsidy during childhood</p> <p>The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR</p> <p>The condition must have genetic confirmation of 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; AND</p> <p>Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug.</p> <p>Patient must be at least 19 years of age at the time of this authority application, but never claimed PBS subsidy for a disease modifying treatment during childhood; AND</p> <p>Patient must have SMA where the onset of signs/symptoms (at least one) of SMA first occurred prior to their 19th birthday (SMA symptom onset after this age will be considered type IV SMA, which is not PBS-subsidised).</p> <p>Must be treated by a specialist medical practitioner experienced in the diagnosis/management of SMA; OR</p> <p>Must be treated by a medical practitioner who has been directed to prescribe this benefit by a specialist medical practitioner experienced in the diagnosis/management of SMA; AND</p>	

C14370	<p>Patient must be undergoing initial PBS-subsidised treatment for untreated disease - prescribe up to 3 repeat prescriptions to enable dosing occurring at days: 0 (original prescription), 14 (repeat 1), 28 (repeat 2), 63 (repeat 3) (i.e. the loading doses); OR</p> <p>Patient must be undergoing initial PBS-subsidised treatment, but the patient has initiated treatment via non-PBS supply (e.g. clinical trial, sponsor compassionate access) - prescribe zero repeat prescriptions where loading doses are complete; AND</p> <p>Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment.</p> <p>The authority application must be made in writing and must include:</p> <p>(1) a completed authority prescription form; and</p> <p>(2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).</p> <p>Signs and symptoms of spinal muscular atrophy in the context of this PBS restriction are:</p> <p>(i) Failure to meet or regression in ability to perform age-appropriate motor milestones,</p> <p>(ii) Proximal weakness,</p> <p>(iii) Hypotonia,</p> <p>(iv) Absence of deep tendon reflexes,</p> <p>(v) Failure to gain weight appropriate for age,</p> <p>(vi) Any active denervation or chronic neurogenic changes found on electromyography,</p> <p>(vii) A compound muscle action potential below normative values for an age-matched child.</p> <p>In this authority application, confirm:</p> <p>(1) the patient's medical history is consistent with a diagnosis of childhood onset spinal muscular atrophy,</p> <p>(2) which of the above (i to vii) (at least 1) were present during childhood,</p> <p>(3) the age of the patient (rounded to the nearest year) when the first sign/symptom was observed.</p> <p>Spinal muscular atrophy (SMA)</p> <p>Changing the prescribed therapy</p> <p>Patient must be undergoing a change in prescribed SMA drug to this drug - the drug treatment being replaced was a PBS benefit initiated after the patient's 19th birthday; AND</p> <p>Must be treated by a specialist medical practitioner experienced in the diagnosis/management of SMA; OR</p> <p>Must be treated by a medical practitioner who has been directed to prescribe this benefit by a specialist medical practitioner experienced in the diagnosis/management of SMA; AND</p> <p>Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment.</p> <p>Patient must be untreated with gene therapy; AND</p> <p>Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug.</p> <p>Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.</p>	Compliance with Authority Required procedures
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C14421	<p>The prescriber has given consideration to whether a 'wash out' period is recommended or not prior to changing the prescribed therapy.</p> <p>Symptomatic type IIIB/IIIC spinal muscular atrophy (SMA) Changing the prescribed therapy</p> <p>Patient must be undergoing a change in prescribed SMA drug to this drug - the drug treatment being replaced was a PBS benefit initiated prior to the patient's 19th birthday for SMA type IIIB/IIIC; AND</p> <p>Must be treated by a specialist medical practitioner experienced in the diagnosis/management of SMA; OR</p> <p>Must be treated by a medical practitioner who has been directed to prescribe this benefit by a specialist medical practitioner experienced in the diagnosis/management of SMA; AND</p> <p>Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment.</p> <p>Patient must be untreated with gene therapy; AND</p> <p>Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug.</p> <p>Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.</p> <p>The prescriber has given consideration to whether a 'wash out' period is recommended or not prior to changing the prescribed therapy.</p>	Compliance with Authority Required procedures
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(b) insert in the column headed "Listed Drug" for the entry for Circumstances Code "C14433": Nusinersen

(c) omit:

C15066	<p>Pre-symptomatic spinal muscular atrophy (SMA) Initial treatment of pre-symptomatic spinal muscular atrophy (SMA) with 1 or 2 copies of the SMN2 gene - Loading doses</p> <p>Must be treated by 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; 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.</p> <p>The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR</p> <p>The condition must have genetic confirmation of 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; AND</p> <p>The condition must be pre-symptomatic SMA, with genetic confirmation that there are 1 to 2 copies of the survival motor neuron 2 (SMN2) gene; AND</p> <p>The treatment must be given concomitantly with best supportive care for this condition; AND</p> <p>The treatment must not exceed four loading doses (at days 0, 14, 28 and 63) under this</p>	Compliance with Written Authority Required procedures
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restriction; AND
 Patient must be untreated with gene therapy.
 Patient must be aged under 36 months prior to commencing treatment.
 Application for authorisation of initial treatment must be in writing (lodged via postal service or electronic upload) and must include:
 (a) a completed authority prescription form; and
 (b) a completed Spinal muscular atrophy PBS Authority Application Form which includes the following:
 (i) confirmation of genetic diagnosis of SMA; and
 (ii) a copy of the results substantiating the number of SMN2 gene copies determined by quantitative polymerase chain reaction (qPCR) or multiple ligation dependent probe amplification (MLPA)

(d) omit:

C15116

Pre-symptomatic spinal muscular atrophy (SMA)
 Initial treatment of pre-symptomatic spinal muscular atrophy (SMA) with 3 copies of the SMN2 gene - Loading doses
 Must be treated by 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; 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.
 The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR
 The condition must have genetic confirmation of 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;
 AND
 The condition must be pre-symptomatic SMA, with genetic confirmation that there are 3 copies of the survival motor neuron 2 (SMN2) gene; AND
 The treatment must be given concomitantly with best supportive care for this condition; AND
 The treatment must not exceed four loading doses (at days 0, 14, 28 and 63) under this restriction; AND
 Patient must be untreated with gene therapy.
 Patient must be aged under 36 months prior to commencing treatment.
 Application for authorisation of initial treatment must be in writing (lodged via postal service or electronic upload) and must include:
 (a) a completed authority prescription form; and
 (b) a completed Spinal muscular atrophy PBS Authority Application Form which includes the following:
 (i) confirmation of genetic diagnosis of SMA; and
 (ii) a copy of the results substantiating the number of SMN2 gene copies determined by quantitative polymerase chain reaction (qPCR) or multiple ligation dependent probe amplification (MLPA)

Compliance with Written Authority Required procedures

(e) insert in numerical order after existing text:

C18053	<p>Spinal muscular atrophy (SMA)</p> <p>Initial treatment occurring after onasemnogene abeparvovec therapy in a patient with one of: (i) Type 1 SMA, or, (ii) pre-symptomatic SMA</p> <p>Patient must have experienced a regression in a developmental state listed below (see 'Definition') despite treatment with gene therapy - confirm that this: (i) not due to an acute concomitant illness; (ii) not due to non-compliance to best-supportive care, (iii) apparent for at least 3 months, (iv) verified by another clinician in the treatment team - state the full name of this clinician plus their profession (e.g. medical practitioner, nurse, physiotherapist; this is not an exhaustive list of examples); AND</p> <p>The treatment must not be a PBS-subsidised benefit where the condition has progressed to a point where invasive permanent assisted ventilation (i.e. ventilation via tracheostomy tube for at least 16 hours per day) is required in the absence of potentially reversible causes; AND</p> <p>The treatment must be given concomitantly with best supportive care for this condition; AND</p> <p>The treatment must not be in combination with PBS-subsidised treatment with risdiplam for this condition.</p> <p>Must be treated by 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; 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; AND</p> <p>Patient must be undergoing treatment under this Treatment phase listing once only - for continuing treatment beyond this authority application, refer to the drug's relevant 'Continuing treatment' listing for the patient's SMA type.</p> <p>Patient must have a prior authority approval for any drug PBS-listed for symptomatic Type 1 SMA, with at least one approval having been for gene therapy; OR</p> <p>Patient must have a prior authority approval for any drug PBS-listed for pre-symptomatic SMA, with at least one approval having been for gene therapy.</p> <p>The authority application must be made in writing and must include:</p> <p>(1) details of the proposed prescription; and</p> <p>(2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).</p> <p>Do not resubmit previously submitted documentation concerning the diagnosis and type of SMA.</p> <p>Confirm that a previous PBS authority application has been approved for one of the following:</p> <p>(i) Symptomatic Type 1 SMA; or</p> <p>(ii) Pre-symptomatic SMA treated with nusinersen.</p> <p>Definition:</p> <p>Various childhood developmental states (1 to 9) are listed below, some followed by further</p>	Compliance with Written Authority Required procedures
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observations (a up to d). Where at least one developmental state/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 (e.g. 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 (e.g. 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.

Confirm which developmental state has regressed by: (i) stating the overall developmental state (1 - 9) the patient was in at the time of gene therapy, or, the best developmental state achieved since gene therapy, and (ii) stating the patient's current overall developmental state (i.e. a

C18073	<p>number that is lower than stated in (i).</p> <p>Where the patient has neither regressed from a developmental state nor reached the next developmental state, PBS-subsidy of this benefit is not available.</p> <p>Symptomatic Type I, II or IIIa spinal muscular atrophy (SMA)</p> <p>Initial treatment - Loading doses</p> <p>Must be treated by 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; 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.</p> <p>The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR</p> <p>The condition must have genetic confirmation of 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; AND</p> <p>Patient must have experienced at least two of the defined signs and symptoms of SMA type I, II or IIIa prior to 3 years of age; AND</p> <p>The treatment must not be in combination with PBS-subsidised treatment with risdiplam for this condition; AND</p> <p>The treatment must be given concomitantly with best supportive care for this condition; AND</p> <p>The treatment must not exceed four loading doses (at days 0, 14, 28 and 63) under this restriction; AND</p> <p>Patient must not have received PBS-subsidised gene therapy.</p> <p>Patient must be 18 years of age or under.</p> <p>Defined signs and symptoms of type I SMA are:</p> <ul style="list-style-type: none"> i) Onset before 6 months of age; and ii) Failure to meet or regression in ability to perform age-appropriate motor milestones; or iii) Proximal weakness; or iv) Hypotonia; or v) Absence of deep tendon reflexes; or vi) Failure to gain weight appropriate for age; or vii) Any active chronic neurogenic changes; or viii) A compound muscle action potential below normative values for an age-matched child. <p>Defined signs and symptoms of type II SMA are:</p> <ul style="list-style-type: none"> i) Onset between 6 and 18 months; and ii) Failure to meet or regression in ability to perform age-appropriate motor milestones; or 	Compliance with Written Authority Required procedures
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C18076	<p>iii) Proximal weakness; or iv) Weakness in trunk righting/derotation; or v) Hypotonia; or vi) Absence of deep tendon reflexes; or vii) Failure to gain weight appropriate for age; or viii) Any active chronic neurogenic changes; or ix) A compound muscle action potential below normative values for an age-matched child. Defined signs and symptoms of type IIIa SMA are: i) Onset between 18 months and 3 years of age; and ii) Failure to meet or regression in ability to perform age-appropriate motor milestones; or iii) Proximal weakness; or iv) Hypotonia; or v) Absence of deep tendon reflexes; or vi) Failure to gain weight appropriate for age; or vii) Any active chronic neurogenic changes; or viii) A compound muscle action potential below normative values for an age-matched child. Application for authorisation of initial treatment must be in writing and must include: (a) details of the proposed prescription; and (b) a completed Spinal muscular atrophy PBS Authority Application Form which includes the following: i) specification of SMA type (I, II or IIIa); and (ii) sign(s) and symptom(s) that the patient has experienced; and (iii) patient's age at the onset of sign(s) and symptom(s).</p>	Compliance with Written Authority Required procedures
	<p>Pre-symptomatic spinal muscular atrophy (SMA) Initial treatment of pre-symptomatic spinal muscular atrophy (SMA) with 3 copies of the SMN2 gene - Loading doses Must be treated by 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; 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. The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR The condition must have genetic confirmation of 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; AND</p>	

C18077	<p>The condition must be pre-symptomatic SMA, with genetic confirmation that there are 3 copies of the survival motor neuron 2 (SMN2) gene; AND</p> <p>The treatment must be given concomitantly with best supportive care for this condition; AND</p> <p>The treatment must not exceed four loading doses (at days 0, 14, 28 and 63) under this restriction; AND</p> <p>Patient must not have received PBS-subsidised gene therapy.</p> <p>Patient must be aged under 36 months prior to commencing treatment.</p> <p>Application for authorisation of initial treatment must be in writing (lodged via postal service or electronic upload) and must include:</p> <p>(a) details of the proposed prescription; and</p> <p>(b) a completed Spinal muscular atrophy PBS Authority Application Form which includes the following:</p> <p>(i) confirmation of genetic diagnosis of SMA; and</p> <p>(ii) a copy of the results substantiating the number of SMN2 gene copies determined by quantitative polymerase chain reaction (qPCR) or multiple ligation dependent probe amplification (MLPA)</p> <p>Symptomatic type IIIB/IIIC spinal muscular atrophy (SMA)</p> <p>Initial PBS-subsidised treatment in a child</p> <p>The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR</p> <p>The condition must have genetic confirmation of 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; AND</p> <p>Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug.</p> <p>Patient must be of an age that is prior to their 19th birthday at the time of this authority application; AND</p> <p>Patient must have SMA type III where the onset of signs/symptoms of SMA first occurred after their 3rd birthday, but before their 19th birthday (SMA type IIIB/IIIC).</p> <p>Must be treated by 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; 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; AND</p> <p>Patient must be undergoing initial PBS-subsidised treatment for untreated disease - prescribe up to 3 repeat prescriptions to enable dosing occurring at days: 0 (original prescription), 14 (repeat 1), 28 (repeat 2), 63 (repeat 3) (i.e. the loading doses); OR</p> <p>Patient must be undergoing initial PBS-subsidised treatment, but the patient has initiated</p>	Compliance with Written Authority Required procedures
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	<p>treatment via non-PBS supply (e.g. clinical trial, sponsor compassionate access) - prescribe zero repeat prescriptions where loading doses are complete; AND</p> <p>Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment.</p> <p>The authority application must be made in writing and must include:</p> <p>(1) details of the proposed prescription; and</p> <p>(2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).</p> <p>Signs and symptoms of spinal muscular atrophy in the context of this PBS restriction are:</p> <p>(i) Failure to meet or regression in ability to perform age-appropriate motor milestones,</p> <p>(ii) Proximal weakness,</p> <p>(iii) Hypotonia,</p> <p>(iv) Absence of deep tendon reflexes,</p> <p>(v) Any active denervation or chronic neurogenic changes found on electromyography,</p> <p>(vi) A compound muscle action potential below normative values for an age-matched child.</p> <p>In this authority application, confirm:</p> <p>(1) the patient's medical history is consistent with a diagnosis of type IIIB/IIIC spinal muscular atrophy,</p> <p>(2) which of the above (i to vi) (at least 1) were present after their 3rd birthday, but before their 19th birthday,</p> <p>(3) the age of the patient (rounded to the nearest year) when the first sign/symptom was observed.</p>	
C18078	<p>Spinal muscular atrophy (SMA)</p> <p>Changing the prescribed therapy</p> <p>Patient must be undergoing a change in prescribed SMA drug to this drug - the drug treatment being replaced was a PBS benefit initiated after the patient's 19th birthday; AND</p> <p>Must be treated by a specialist medical practitioner experienced in the diagnosis/management of SMA; OR</p> <p>Must be treated by a medical practitioner who has been directed to prescribe this benefit by a specialist medical practitioner experienced in the diagnosis/management of SMA; AND</p> <p>Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment.</p> <p>Patient must not have received PBS-subsidised gene therapy; AND</p> <p>Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug.</p> <p>Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.</p>	Compliance with Authority Required procedures

C18094	<p>The prescriber has given consideration to whether a 'wash out' period is recommended or not prior to changing the prescribed therapy.</p> <p>Spinal muscular atrophy (SMA)</p> <p>Initial PBS-subsidised treatment in an adult who did not initiate PBS subsidy during childhood</p> <p>The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR</p> <p>The condition must have genetic confirmation of 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; AND</p> <p>Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug.</p> <p>Patient must be at least 19 years of age at the time of this authority application, but never claimed PBS subsidy for a disease modifying treatment during childhood; AND</p> <p>Patient must have SMA where the onset of signs/symptoms (at least one) of SMA first occurred prior to their 19th birthday (SMA symptom onset after this age will be considered type IV SMA, which is not PBS-subsidised).</p> <p>Must be treated by a specialist medical practitioner experienced in the diagnosis/management of SMA; OR</p> <p>Must be treated by a medical practitioner who has been directed to prescribe this benefit by a specialist medical practitioner experienced in the diagnosis/management of SMA; AND</p> <p>Patient must be undergoing initial PBS-subsidised treatment for untreated disease - prescribe up to 3 repeat prescriptions to enable dosing occurring at days: 0 (original prescription), 14 (repeat 1), 28 (repeat 2), 63 (repeat 3) (i.e. the loading doses); OR</p> <p>Patient must be undergoing initial PBS-subsidised treatment, but the patient has initiated treatment via non-PBS supply (e.g. clinical trial, sponsor compassionate access) - prescribe zero repeat prescriptions where loading doses are complete; AND</p> <p>Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment.</p> <p>The authority application must be made in writing and must include:</p> <p>(1) details of the proposed prescription; and</p> <p>(2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).</p> <p>Signs and symptoms of spinal muscular atrophy in the context of this PBS restriction are:</p> <p>(i) Failure to meet or regression in ability to perform age-appropriate motor milestones,</p> <p>(ii) Proximal weakness,</p> <p>(iii) Hypotonia,</p> <p>(iv) Absence of deep tendon reflexes,</p> <p>(v) Failure to gain weight appropriate for age,</p>	Compliance with Written Authority Required procedures
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C18101	<p>(vi) Any active denervation or chronic neurogenic changes found on electromyography, (vii) A compound muscle action potential below normative values for an age-matched child. In this authority application, confirm: (1) the patient's medical history is consistent with a diagnosis of childhood onset spinal muscular atrophy, (2) which of the above (i to vii) (at least 1) were present during childhood, (3) the age of the patient (rounded to the nearest year) when the first sign/symptom was observed.</p> <p>Symptomatic type IIIB/IIIC spinal muscular atrophy (SMA) Changing the prescribed therapy</p> <p>Patient must be undergoing a change in prescribed SMA drug to this drug - the drug treatment being replaced was a PBS benefit initiated prior to the patient's 19th birthday for SMA type IIIB/IIIC; AND</p> <p>Must be treated by a specialist medical practitioner experienced in the diagnosis/management of SMA; OR</p> <p>Must be treated by a medical practitioner who has been directed to prescribe this benefit by a specialist medical practitioner experienced in the diagnosis/management of SMA; AND</p> <p>Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment.</p> <p>Patient must not have received PBS-subsidised gene therapy; AND</p> <p>Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug.</p> <p>Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.</p> <p>The prescriber has given consideration to whether a 'wash out' period is recommended or not prior to changing the prescribed therapy.</p>	Compliance with Authority Required procedures
C18110	<p>Pre-symptomatic spinal muscular atrophy (SMA)</p> <p>Initial treatment of pre-symptomatic spinal muscular atrophy (SMA) with 1 or 2 copies of the SMN2 gene - Loading doses</p> <p>Must be treated by 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; 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.</p> <p>The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR</p> <p>The condition must have genetic confirmation of 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;</p>	Compliance with Written Authority Required procedures

AND

The condition must be pre-symptomatic SMA, with genetic confirmation that there are 1 to 2 copies of the survival motor neuron 2 (SMN2) gene; AND

The treatment must be given concomitantly with best supportive care for this condition; AND

The treatment must not exceed four loading doses (at days 0, 14, 28 and 63) under this restriction; AND

Patient must not have received PBS-subsidised gene therapy.

Patient must be aged under 36 months prior to commencing treatment.

Application for authorisation of initial treatment must be in writing (lodged via postal service or electronic upload) and must include:

(a) details of the proposed prescription; and

(b) a completed Spinal muscular atrophy PBS Authority Application Form which includes the following:

(i) confirmation of genetic diagnosis of SMA; and

(ii) a copy of the results substantiating the number of SMN2 gene copies determined by quantitative polymerase chain reaction (qPCR) or multiple ligation dependent probe amplification (MLPA)

[10] Schedule 3, entry for Ravulizumab

insert in numerical order after existing text:

C18042

Bridging therapy for generalised myasthenia gravis (gMG)

Non-loading/maintenance doses

Patient must have a diagnosis of MGFA Disease Class II to IV; AND

Patient must have a positive serology for acetylcholine receptor (AChR) binding autoantibodies; AND

Patient must not be experiencing a myasthenic crisis; AND

Patient must be receiving concomitant treatment with a non-steroidal immunosuppressant (NS-IST); OR

Patient must have had a thymectomy; AND

Patient must be receiving concomitant treatment with an oral corticosteroid; AND

Patient must have a MG-ADL score of 6 (or higher) and a MGC score of 10 (or higher), despite having undergone 2 of the following 3 remission inducing treatments: (i) NS-IST for 3 months; (ii) oral corticosteroids for 3 months; (iii) a thymectomy; AND

Patient must not be receiving concomitant treatment with any of the following: (i) another gMG biological agent, (ii) intravenous immunoglobulin (IVIg), (iii) plasma exchange (PLEX), (iv) rituximab for this condition; AND

Patient must not receive more than 6 months of bridging treatment with any gMG biological

Compliance with Authority
Required procedures

C18043	<p>agent under this PBS indication.</p> <p>Must be treated by a prescriber who is either: (i) a neurologist; (ii) a clinical immunologist with expertise in the treatment of myasthenia gravis; OR</p> <p>Must be treated by a medical practitioner who has consulted at least one of the above mentioned specialist types, with an agreement reached that the patient should be treated with this pharmaceutical benefit on this occasion.</p> <p>At the time of authority application, medical practitioners must request the appropriate number of vials to provide sufficient dose of the drug, according to the specified dosage in the TGA approved Product Information (PI).</p> <p>An appropriate amount of drug (maximum quantity in units) may require prescribing both strengths. Consider strengths and combinations that minimise drug wastage. A separate authority prescription form must be completed for each strength requested.</p> <p>Applications for treatment with this drug where the dose and dosing frequency exceeds that specified in the TGA PI will not be approved.</p> <p>The authority application must be via the Online PBS Authorities System or in writing and must include:</p> <p>(a) the MG-ADL and MGC score after 3-months of remission-inducing treatments (include the date the assessments were conducted);</p> <p>(b) details of remission-inducing treatments [date commencement and duration of drug therapy including drug names and dosages, and/or date of the thymectomy].</p> <p>If the application is submitted through HPOS form upload or mail, it must include the following:</p> <p>(1) details of the proposed prescription; and</p> <p>(2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).</p> <p>A retrospective assessment for one of the MGC score or MG-ADL score can be accepted in cases where it was not conducted after completing 3 months of remission inducing treatments.</p> <p>Treatment refractory generalised myasthenia gravis (gMG)</p> <p>Initial treatment - Treatment refractory gMG patients (Non-loading/maintenance doses)</p> <p>Patient must have a diagnosis of MGFA Disease Class II to IV; AND</p> <p>Patient must have a positive serology for acetylcholine receptor (AChR) binding autoantibodies; AND</p> <p>Patient must not be experiencing a myasthenic crisis; AND</p> <p>Patient must not have received treatment with a gMG biologic within 3 months prior to the first authority application for this indication (i.e. in treatment refractory setting); OR</p> <p>Patient must be considered by the treating clinician to have deteriorating gMG disease during a treatment break with a gMG biological agent; AND</p> <p>Patient must not be receiving concomitant treatment with any of the following: (i) another gMG biological agent, (ii) intravenous immunoglobulin (IVIg), (iii) plasma exchange (PLEX), (iv)</p>	Compliance with Authority Required procedures
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rituximab for this condition; AND
 Patient must be receiving concomitant treatment with a non-steroidal immunosuppressant (NS-IST); OR
 Patient must have had a thymectomy; AND
 Patient must have a MG-ADL score of 6 (or higher) and a MGC score of 10 (or higher), despite having undergone 2 of the following 3 remission inducing treatments: (i) NS-IST for a minimum of 12 months, (ii) oral corticosteroids for a minimum of 12 months, (iii) a thymectomy; AND
 Patient must provide no more than 6 months of therapy per initial authority application.
 Must be treated by a prescriber who is either: (i) a neurologist; (ii) a clinical immunologist with expertise in the treatment of myasthenia gravis; OR
 Must be treated by a medical practitioner who has consulted at least one of the above mentioned specialist types, with an agreement reached that the patient should be treated with this pharmaceutical benefit on this occasion.
 Patients who are considered to have deteriorating gMG disease while on a treatment break with a gMG biologic may qualify with 9 months of remission inducing treatments (rather than 12 months).
 At the time of authority application, medical practitioners must request the appropriate number of vials to provide sufficient dose of the drug, according to the specified dosage in the TGA approved Product Information (PI).
 An appropriate amount of drug (maximum quantity in units) may require prescribing both strengths. Consider strengths and combinations that minimise drug wastage. A separate authority prescription form must be completed for each strength requested.
 Applications for treatment with this drug where the dose and dosing frequency exceeds that specified in the TGA PI will not be approved.
 The authority application must be via the Online PBS Authorities System, or in writing and must include:
 (a) details of remission-inducing treatments [date commencement and duration of drug therapy including drug names and dosages, and/or date of the thymectomy];
 (b) the baseline MG-ADL and MGC scores assessed after completing the 12 months of remission-inducing treatments (include the date the assessments were conducted).
 If the application is submitted through HPOS form upload or mail, it must include:
 (1) details of the proposed prescription; and
 (2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).

C18044

Treatment refractory generalised myasthenia gravis (gMG)
 Initial treatment - Treatment refractory gMG patients (Loading dose)
 Patient must have a diagnosis of MGFA Disease Class II to IV; AND
 Patient must have a positive serology for acetylcholine receptor (AChR) binding autoantibodies;

Compliance with Authority
 Required procedures

AND

Patient must not be experiencing a myasthenic crisis; AND

Patient must not have received treatment with a gMG biologic within 3 months prior to the first authority application for this indication (i.e. in treatment refractory setting); OR

Patient must be considered by the treating clinician to have deteriorating gMG disease during a treatment break with a gMG biological agent; AND

Patient must not be receiving concomitant treatment with any of the following: (i) another gMG biological agent, (ii) intravenous immunoglobulin (IVIg), (iii) plasma exchange (PLEX), (iv) rituximab for this condition; AND

Patient must be receiving concomitant treatment with a non-steroidal immunosuppressant (NS-IST); OR

Patient must have had a thymectomy; AND

Patient must have a MG-ADL score of 6 (or higher) and a MGC score of 10 (or higher), despite having undergone 2 of the following 3 remission inducing treatments: (i) NS-IST for a minimum of 12 months, (ii) oral corticosteroids for a minimum of 12 months, (iii) a thymectomy.

Must be treated by a prescriber who is either: (i) a neurologist; (ii) a clinical immunologist with expertise in the treatment of myasthenia gravis; OR

Must be treated by a medical practitioner who has consulted at least one of the above mentioned specialist types, with an agreement reached that the patient should be treated with this pharmaceutical benefit on this occasion.

Patients who are considered to have deteriorating gMG disease while on a treatment break with a gMG biologic may qualify with 9 months of remission inducing treatments (rather than 12 months).

Where loading doses of ravulizumab are required, additional authority prescriptions may be requested and approved in the same day.

At the time of authority application, medical practitioners must request the appropriate number of vials for a single loading dose based on the patient's weight, according to the specified dosage in the TGA approved Product Information (PI).

An appropriate amount of drug (maximum quantity in units) may require prescribing both strengths. Consider strengths and combinations that minimise drug wastage. A separate authority prescription form must be completed for each strength requested.

Applications for treatment with this drug where the dose and dosing frequency exceeds that specified in the TGA PI will not be approved.

The authority application must be via the Online PBS Authorities System, or in writing and must include:

(a) details of remission-inducing treatments [date commencement and duration of drug therapy including drug names and dosages, and/or date of the thymectomy];

(b) the baseline MG-ADL and MGC scores assessed after completing the 12 months of remission-inducing treatments (include the date the assessments were conducted).

C18045	<p>If the application is submitted through HPOS form upload or mail, it must include:</p> <p>(1) details of the proposed prescription; and</p> <p>(2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).</p> <p>Acute severe generalised myasthenia gravis (gMG)</p> <p>Non-loading/maintenance doses</p> <p>Patient must have a diagnosis of MGFA Disease Class II to IV; AND</p> <p>Patient must have a positive serology for acetylcholine receptor (AChR) binding autoantibodies; AND</p> <p>Patient must not be experiencing a myasthenic crisis; AND</p> <p>Patient must be considered by the treating clinician to have rapidly deteriorating gMG disease in the absence of immediate treatment with a gMG biological agent; AND</p> <p>Patient must not be receiving concomitant treatment with any of the following: (i) another gMG biological agent, (ii) intravenous immunoglobulin (IVIg), (iii) plasma exchange (PLEX), (iv) rituximab for this condition; AND</p> <p>Patient must be receiving concomitant treatment with a non-steroidal immunosuppressant (NS-IST); OR</p> <p>Patient must be commencing treatment with an NS-IST within 2 weeks; OR</p> <p>Patient must have had a thymectomy; AND</p> <p>Patient must be receiving concomitant treatment with an oral corticosteroid; AND</p> <p>Patient must not receive more than 3 months of total treatment with gMG biological agents under this PBS indication ('acute severe gMG'); AND</p> <p>Patient must not have accessed a prior PBS-subsidised gMG biological agent; OR</p> <p>Patient must have received a loading dose of this drug for this indication.</p> <p>Must be treated by a prescriber who is either: (i) a neurologist; (ii) a clinical immunologist with expertise in the treatment of myasthenia gravis; OR</p> <p>Must be treated by a medical practitioner who has consulted at least one of the above mentioned specialist types, with an agreement reached that the patient should be treated with this pharmaceutical benefit on this occasion.</p> <p>At the time of authority application, medical practitioners must request the appropriate number of vials to provide sufficient dose of the drug, according to the specified dosage in the TGA approved Product Information (PI).</p> <p>An appropriate amount of drug (maximum quantity in units) may require prescribing both strengths. Consider strengths and combinations that minimise drug wastage. A separate authority prescription form must be completed for each strength requested.</p> <p>Applications for treatment with this drug where the dose and dosing frequency exceeds that specified in the TGA PI will not be approved.</p> <p>The authority application must be via the Online PBS Authorities System or in writing via HPOS</p>	Compliance with Authority Required procedures
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C18046	<p>form upload or mail.</p> <p>If the application is submitted through HPOS form upload or mail, it must include the following:</p> <p>(1) details of the proposed prescription; and</p> <p>(2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).</p> <p>Acute severe generalised myasthenia gravis (gMG)</p> <p>Loading dose</p> <p>Patient must have a diagnosis of MGFA Disease Class II to IV; AND</p> <p>Patient must have a positive serology for acetylcholine receptor (AChR) binding autoantibodies; AND</p> <p>Patient must not be experiencing a myasthenic crisis; AND</p> <p>Patient must be considered by the treating clinician to have rapidly deteriorating gMG disease in the absence of immediate treatment with a gMG biological agent; AND</p> <p>Patient must not be receiving concomitant treatment with any of the following: (i) another gMG biological agent, (ii) intravenous immunoglobulin (IVIg), (iii) plasma exchange (PLEX), (iv) rituximab for this condition; AND</p> <p>Patient must be receiving concomitant treatment with a non-steroidal immunosuppressant (NS-IST); OR</p> <p>Patient must be commencing treatment with an NS-IST within 2 weeks; OR</p> <p>Patient must have had a thymectomy; AND</p> <p>Patient must be receiving concomitant treatment with an oral corticosteroid; AND</p> <p>Patient must not receive more than 3 months of total treatment with gMG biological agents under this PBS indication ('acute severe gMG'); AND</p> <p>Patient must not have accessed a prior PBS-subsidised gMG biological agent.</p> <p>Must be treated by a prescriber who is either: (i) a neurologist; (ii) a clinical immunologist with expertise in the treatment of myasthenia gravis; OR</p> <p>Must be treated by a medical practitioner who has consulted at least one of the above mentioned specialist types, with an agreement reached that the patient should be treated with this pharmaceutical benefit on this occasion.</p> <p>Where loading doses of ravulizumab are required, additional authority prescriptions may be requested and approved in the same day.</p> <p>At the time of authority application, medical practitioners must request the appropriate number of vials for a single loading dose based on the patient's weight, according to the specified dosage in the TGA approved Product Information (PI).</p> <p>An appropriate amount of drug (maximum quantity in units) may require prescribing both strengths. Consider strengths and combinations that minimise drug wastage. A separate authority prescription form must be completed for each strength requested.</p> <p>Applications for treatment with this drug where the dose and dosing frequency exceeds that</p>	Compliance with Authority Required procedures
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C18057	<p>specified in the TGA PI will not be approved.</p> <p>The authority application must be via the Online PBS Authorities System or in writing via HPOS form upload or mail.</p> <p>If the application is submitted through HPOS form upload or mail, it must include the following:</p> <p>(1) details of the proposed prescription; and</p> <p>(2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).</p> <p>Treatment refractory generalised myasthenia gravis (gMG)</p> <p>Continuing treatment - Treatment refractory gMG patients</p> <p>Patient must have previously received PBS-subsidised treatment with a gMG biological agent for this PBS indication; AND</p> <p>Patient must have demonstrated a clinical improvement based on a decrease in MG-ADL score of at least 2 points from baseline; AND</p> <p>Patient must have demonstrated a clinical improvement based on a decrease in MGC score of at least 3 points from baseline; AND</p> <p>Patient must not be receiving concomitant treatment with any of the following: (i) another gMG biological agent, (ii) intravenous immunoglobulin (IVIg), (iii) plasma exchange (PLEX), (iv) rituximab for this condition; AND</p> <p>Patient must be receiving concomitant treatment with a non-steroidal immunosuppressant (NS-IST); OR</p> <p>Patient must have had a thymectomy; AND</p> <p>The treatment must provide no more than 6 months of therapy per continuing authority application.</p> <p>Must be treated by a prescriber who is either: (i) a neurologist; (ii) a clinical immunologist with expertise in the treatment of myasthenia gravis; OR</p> <p>Must be treated by a medical practitioner who has consulted at least one of the above mentioned specialist types, with an agreement reached that the patient should be treated with this pharmaceutical benefit on this occasion.</p> <p>At the time of authority application, medical practitioners must request the appropriate number of vials to provide sufficient dose of the drug, according to the specified dosage in the TGA approved Product Information (PI).</p> <p>An appropriate amount of drug (maximum quantity in units) may require prescribing both strengths. Consider strengths and combinations that minimise drug wastage. A separate authority prescription form must be completed for each strength requested.</p> <p>Applications for treatment with this drug where the dose and dosing frequency exceeds that specified in the TGA PI will not be approved.</p> <p>The authority application must be via the Online PBS Authorities System, or in writing and must include the MG-ADL and MGC scores assessed from the most recent course of treatment.</p>	Compliance with Authority Required procedures
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C18098	<p>If the application is submitted through HPOS form upload or mail, it must include:</p> <p>(1) details of the proposed prescription; and</p> <p>(2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).</p> <p>Treatment refractory generalised myasthenia gravis (gMG)</p> <p>Transitioning from non-PBS to PBS-subsidised treatment - Grandfathering treatment</p> <p>Patient must have received non-PBS-subsidised treatment with this drug for this PBS indication prior to 1 March 2026; AND</p> <p>Patient must have a diagnosis of MGFA Disease Class II to IV; AND</p> <p>Patient must have a positive serology for acetylcholine receptor (AChR) binding autoantibodies; AND</p> <p>Patient must not be experiencing a myasthenic crisis; AND</p> <p>Patient must not be receiving concomitant treatment with any of the following: (i) another gMG biological agent, (ii) intravenous immunoglobulin (IVIg), (iii) plasma exchange (PLEX), (iv) rituximab for this condition; AND</p> <p>Patient must be receiving concomitant treatment with a non-steroidal immunosuppressant (NS-IST); OR</p> <p>Patient must have had a thymectomy; AND</p> <p>Patient must have had a MG-ADL score of 6 (or higher) and a MGC score of 10 (or higher), despite having undergone 2 of the following 3 remission inducing treatments: (i) NS-IST for a minimum of 12 months, (ii) oral corticosteroids for a minimum of 12 months, (iii) a thymectomy; AND</p> <p>Patient must have demonstrated a clinical improvement based on a decrease in MG-ADL score of at least 2 points from baseline; AND</p> <p>Patient must have demonstrated a clinical improvement based on a decrease in MGC score of at least 3 points from baseline; AND</p> <p>The treatment must provide no more than 6 months of therapy under this restriction.</p> <p>Must be treated by a prescriber who is either: (i) a neurologist; (ii) a clinical immunologist with expertise in the treatment of myasthenia gravis; OR</p> <p>Must be treated by a medical practitioner who has consulted at least one of the above mentioned specialist types, with an agreement reached that the patient should be treated with this pharmaceutical benefit on this occasion.</p> <p>The authority application must be via the Online PBS Authorities System, or in writing and must include:</p> <p>(a) details of remission-inducing treatments [date commencement and duration of drug therapy including drug names and dosages, and/or date of the thymectomy];</p> <p>(b) the baseline MG-ADL and MGC scores assessed after completing the 12 months of remission-inducing treatments (include the date the assessment was conducted);</p>	Compliance with Authority Required procedures
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C18103	<p>(c) the MG-ADL and MGC scores assessed from the most recent course of treatment demonstrating clinical improvement to treatment (include the date the assessment was conducted).</p> <p>If the application is submitted through HPOS form upload or mail, it must include:</p> <p>(1) details of the proposed prescription; and</p> <p>(2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).</p> <p>A retrospective assessment for one of the MGC score or MG-ADL score after completing the 12 months remission inducing treatments can be accepted for grandfathered patients in cases where it was not conducted prior to commencing non-PBS-subsidised treatment for this indication.</p> <p>Bridging therapy for generalised myasthenia gravis (gMG) Loading dose</p> <p>Patient must have a diagnosis of MGFA Disease Class II to IV; AND</p> <p>Patient must have a positive serology for acetylcholine receptor (AChR) binding autoantibodies; AND</p> <p>Patient must not be experiencing a myasthenic crisis; AND</p> <p>Patient must be receiving concomitant treatment with a non-steroidal immunosuppressant (NS-IST); OR</p> <p>Patient must have had a thymectomy; AND</p> <p>Patient must be receiving concomitant treatment with an oral corticosteroid; AND</p> <p>Patient must have a MG-ADL score of 6 (or higher) and a MGC score of 10 (or higher), despite having undergone 2 of the following 3 remission inducing treatments: (i) NS-IST for 3 months; (ii) oral corticosteroids for 3 months; (iii) a thymectomy; AND</p> <p>Patient must not be receiving concomitant treatment with any of the following: (i) another gMG biological agent, (ii) intravenous immunoglobulin (IVIg), (iii) plasma exchange (PLEX), (iv) rituximab for this condition; AND</p> <p>Patient must not receive more than 6 months of bridging treatment with any gMG biological agent under this PBS indication ('bridging therapy for gMG').</p> <p>Must be treated by a prescriber who is either: (i) a neurologist; (ii) a clinical immunologist with expertise in the treatment of myasthenia gravis; OR</p> <p>Must be treated by a medical practitioner who has consulted at least one of the above mentioned specialist types, with an agreement reached that the patient should be treated with this pharmaceutical benefit on this occasion.</p> <p>Where loading doses of ravulizumab are required, additional authority prescriptions may be requested and approved in the same day.</p> <p>At the time of authority application, medical practitioners must request the appropriate number of vials for a single loading dose based on the patient's weight, according to the specified dosage in</p>	Compliance with Authority Required procedures
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the TGA approved Product Information (PI).

An appropriate amount of drug (maximum quantity in units) may require prescribing both strengths. Consider strengths and combinations that minimise drug wastage. A separate authority prescription form must be completed for each strength requested.

Applications for treatment with this drug where the dose and dosing frequency exceeds that specified in the TGA PI will not be approved.

The authority application must be via the Online PBS Authorities System or in writing and must include:

(a) the MG-ADL and MGC score after 3-months of remission-inducing treatments (include the date the assessments were conducted);

(b) details of remission-inducing treatments [date commencement and duration of drug therapy including drug names and dosages, and/or date of the thymectomy].

If the application is submitted through HPOS form upload or mail, it must include the following:

(1) details of the proposed prescription; and

(2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice).

A retrospective assessment for one of the MGC score or MG-ADL score can be accepted in cases where it was not conducted after completing 3 months of remission inducing treatments.

[11] Schedule 3, entry for Risdiplam

(a) *omit:*

C14372	<p>Symptomatic Type I, II or IIIa spinal muscular atrophy (SMA) Initial treatment The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR The condition must have genetic confirmation of 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; AND Patient must have experienced at least two of the defined signs and symptoms of SMA type I, II or IIIa prior to 3 years of age; AND The treatment must be given concomitantly with best supportive care for this condition; AND The treatment must not be in combination with PBS-subsidised treatment with nusinersen for this condition; AND The treatment must be ceased when invasive permanent assisted ventilation is required in the absence of a potentially reversible cause while being treated with this drug. Must be treated by a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic, or in consultation with a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic. Patient must be untreated with gene therapy.</p>	Compliance with Written Authority Required procedures
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Patient must be 18 years of age or under.
Defined signs and symptoms of type I SMA are:
i) Onset before 6 months of age; and
ii) Failure to meet or regression in ability to perform age-appropriate motor milestones; or
iii) Proximal weakness; or
iv) Hypotonia; or
v) Absence of deep tendon reflexes; or
vi) Failure to gain weight appropriate for age; or
vii) Any active chronic neurogenic changes; or
viii) A compound muscle action potential below normative values for an age-matched child.
Defined signs and symptoms of type II SMA are:
i) Onset between 6 and 18 months; and
ii) Failure to meet or regression in ability to perform age-appropriate motor milestones; or
iii) Proximal weakness; or
iv) Weakness in trunk righting/derotation; or
v) Hypotonia; or
vi) Absence of deep tendon reflexes; or
vii) Failure to gain weight appropriate for age; or
viii) Any active chronic neurogenic changes; or
ix) A compound muscle action potential below normative values for an age-matched child.
Defined signs and symptoms of type IIIa SMA are:
i) Onset between 18 months and 3 years of age; and
ii) Failure to meet or regression in ability to perform age-appropriate motor milestones; or
iii) Proximal weakness; or
iv) Hypotonia; or
v) Absence of deep tendon reflexes; or
vi) Failure to gain weight appropriate for age; or
vii) Any active chronic neurogenic changes; or
viii) A compound muscle action potential below normative values for an age-matched child.
Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.
Application for authorisation of initial treatment must be in writing and must include:
(a) a completed authority prescription form; and
(b) a completed Spinal muscular atrophy PBS Authority Application Form which includes the following:
i) specification of SMA type (I, II or IIIa); and
(ii) sign(s) and symptom(s) that the patient has experienced; and
(iii) patient's age at the onset of sign(s) and symptom(s).
The approved Product Information recommended dosing is as follows:
(i) 16 days to less than 2 months of age: 0.15 mg/kg
(ii) 2 months to less than 2 years of age: 0.20 mg/kg
(iii) 2 years of age and older weighing less than 20 kg: 0.25 mg/kg
(iv) 2 years of age and older weighing 20 kg or more: 5 mg
In this authority application, state which of (i) to (iv) above applies to the patient. Based on (i) to (iv), prescribe up to:

1 unit where (i) applies;
 2 units where (ii) applies;
 3 units where (iii) applies;
 3 units where (iv) applies.

(b) omit:

C15990	<p>Pre-symptomatic spinal muscular atrophy (SMA) Initial treatment of pre-symptomatic spinal muscular atrophy (SMA) with 3 copies of the SMN2 gene Must be treated by a specialist medical practitioner experienced in the diagnosis/management of SMA; OR Must be treated by a medical practitioner who has been directed to prescribe this benefit by a specialist medical practitioner experienced in the diagnosis/management of SMA. The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR The condition must have genetic confirmation of 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; AND The condition must be pre-symptomatic SMA, with genetic confirmation that there are 3 copies of the survival motor neuron 2 (SMN2) gene; AND The treatment must be given concomitantly with best supportive care for this condition; AND Patient must be untreated with gene therapy. Patient must be aged under 36 months prior to commencing treatment. Application for authorisation of initial treatment must be in writing (lodged via postal service or electronic upload) and must include: (a) details of the proposed prescription; and (b) a completed Spinal muscular atrophy PBS Authority Application Form which includes the following: (i) confirmation of genetic diagnosis of SMA; and (ii) a copy of the results substantiating the number of SMN2 gene copies determined by quantitative polymerase chain reaction (qPCR) or multiple ligation dependent probe amplification (MLPA) The quantity of drug and number of repeat prescriptions prescribed is to be in accordance with the relevant 'Note' attached to this listing. The approved Product Information recommended dosing is as follows: (i) 16 days to less than 2 months of age: 0.15 mg/kg (ii) 2 months to less than 2 years of age: 0.20 mg/kg (iii) 2 years of age and older weighing less than 20 kg: 0.25 mg/kg</p>	<p>Compliance with Written Authority Required procedures</p>
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C16257	<p>(iv) 2 years of age and older weighing 20 kg or more: 5 mg</p> <p>In this authority application, state which of (i) to (iv) above applies to the patient. Based on (i) to (iv), prescribe up to:</p> <p>1 unit where (i) applies; 2 units where (ii) applies; 3 units where (iii) applies; 3 units where (iv) applies.</p> <p>Pre-symptomatic spinal muscular atrophy (SMA)</p> <p>Initial treatment with this drug of pre-symptomatic spinal muscular atrophy (SMA)</p> <p>Must be treated by a specialist medical practitioner experienced in the diagnosis/management of SMA; OR</p> <p>Must be treated by a medical practitioner who has been directed to prescribe this benefit by a specialist medical practitioner experienced in the diagnosis/management of SMA.</p> <p>The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR</p> <p>The condition must have genetic confirmation of 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; AND</p> <p>The condition must have genetic confirmation that there are 1 to 2 copies of the survival motor neuron 2 (SMN2) gene; AND</p> <p>The condition must be pre-symptomatic; AND</p> <p>The treatment must be given concomitantly with best supportive care for this condition; AND</p> <p>Patient must be untreated with gene therapy.</p> <p>Patient must be aged under 36 months prior to commencing treatment.</p> <p>Application for authorisation of initial treatment must be in writing (lodged via postal service or electronic upload) and must include:</p> <p>(a) details of the proposed prescription; and</p> <p>(b) a completed Spinal muscular atrophy PBS Authority Application Form which includes the following:</p> <p>(i) confirmation of genetic diagnosis of SMA; and</p> <p>(ii) a copy of the results substantiating the number of SMN2 gene copies determined by quantitative polymerase chain reaction (qPCR) or multiple ligation dependent probe amplification (MLPA)</p> <p>The quantity of drug and number of repeat prescriptions prescribed is to be in accordance with the relevant 'Note' attached to this listing.</p> <p>The approved Product Information recommended dosing is as follows:</p> <p>(i) 16 days to less than 2 months of age: 0.15 mg/kg</p>	Compliance with Written Authority Required procedures
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- (ii) 2 months to less than 2 years of age: 0.20 mg/kg
- (iii) 2 years of age and older weighing less than 20 kg: 0.25 mg/kg
- (iv) 2 years of age and older weighing 20 kg or more: 5 mg

In this authority application, state which of (i) to (iv) above applies to the patient. Based on (i) to (iv), prescribe up to:

- 1 unit where (i) applies;
- 2 units where (ii) applies;
- 3 units where (iii) applies;
- 3 units where (iv) applies.

(c) *insert in numerical order after existing text:*

C18087	<p>Symptomatic Type I, II or IIIa spinal muscular atrophy (SMA) Initial treatment</p> <p>The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR</p> <p>The condition must have genetic confirmation of 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; AND</p> <p>Patient must have experienced at least two of the defined signs and symptoms of SMA type I, II or IIIa prior to 3 years of age; AND</p> <p>The treatment must be given concomitantly with best supportive care for this condition; AND</p> <p>The treatment must not be in combination with PBS-subsidised treatment with nusinersen for this condition; AND</p> <p>The treatment must be ceased when invasive permanent assisted ventilation is required in the absence of a potentially reversible cause while being treated with this drug.</p> <p>Must be treated by a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic, or in consultation with a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic.</p> <p>Patient must not have received PBS-subsidised gene therapy.</p> <p>Patient must be 18 years of age or under.</p> <p>Defined signs and symptoms of type I SMA are:</p> <ul style="list-style-type: none"> i) Onset before 6 months of age; and ii) Failure to meet or regression in ability to perform age-appropriate motor milestones; or iii) Proximal weakness; or iv) Hypotonia; or 	<p>Compliance with Written Authority Required procedures</p>
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- v) Absence of deep tendon reflexes; or
- vi) Failure to gain weight appropriate for age; or
- vii) Any active chronic neurogenic changes; or
- viii) A compound muscle action potential below normative values for an age-matched child.

Defined signs and symptoms of type II SMA are:

- i) Onset between 6 and 18 months; and
- ii) Failure to meet or regression in ability to perform age-appropriate motor milestones; or
- iii) Proximal weakness; or
- iv) Weakness in trunk righting/derotation; or
- v) Hypotonia; or
- vi) Absence of deep tendon reflexes; or
- vii) Failure to gain weight appropriate for age; or
- viii) Any active chronic neurogenic changes; or
- ix) A compound muscle action potential below normative values for an age-matched child.

Defined signs and symptoms of type IIIa SMA are:

- i) Onset between 18 months and 3 years of age; and
- ii) Failure to meet or regression in ability to perform age-appropriate motor milestones; or
- iii) Proximal weakness; or
- iv) Hypotonia; or
- v) Absence of deep tendon reflexes; or
- vi) Failure to gain weight appropriate for age; or
- vii) Any active chronic neurogenic changes; or
- viii) A compound muscle action potential below normative values for an age-matched child.

Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.

Application for authorisation of initial treatment must be in writing and must include:

- (a) details of the proposed prescription; and
- (b) a completed Spinal muscular atrophy PBS Authority Application Form which includes the following:
 - i) specification of SMA type (I, II or IIIa); and
 - ii) sign(s) and symptom(s) that the patient has experienced; and
 - iii) patient's age at the onset of sign(s) and symptom(s).

The approved Product Information recommended dosing is as follows:

- (i) 16 days to less than 2 months of age: 0.15 mg/kg
- (ii) 2 months to less than 2 years of age: 0.20 mg/kg

C18090	<p>(iii) 2 years of age and older weighing less than 20 kg: 0.25 mg/kg (iv) 2 years of age and older weighing 20 kg or more: 5 mg In this authority application, state which of (i) to (iv) above applies to the patient. Based on (i) to (iv), prescribe up to: 1 unit where (i) applies; 2 units where (ii) applies; 3 units where (iii) applies; 3 units where (iv) applies.</p> <p>Pre-symptomatic spinal muscular atrophy (SMA) Initial treatment of pre-symptomatic spinal muscular atrophy (SMA) with 3 copies of the SMN2 gene Must be treated by a specialist medical practitioner experienced in the diagnosis/management of SMA; OR Must be treated by a medical practitioner who has been directed to prescribe this benefit by a specialist medical practitioner experienced in the diagnosis/management of SMA. The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR The condition must have genetic confirmation of 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; AND The condition must be pre-symptomatic SMA, with genetic confirmation that there are 3 copies of the survival motor neuron 2 (SMN2) gene; AND The treatment must be given concomitantly with best supportive care for this condition; AND Patient must not have received PBS-subsidised gene therapy. Patient must be aged under 36 months prior to commencing treatment. Application for authorisation of initial treatment must be in writing (lodged via postal service or electronic upload) and must include: (a) details of the proposed prescription; and (b) a completed Spinal muscular atrophy PBS Authority Application Form which includes the following: (i) confirmation of genetic diagnosis of SMA; and (ii) a copy of the results substantiating the number of SMN2 gene copies determined by quantitative polymerase chain reaction (qPCR) or multiple ligation dependent probe amplification (MLPA) The quantity of drug and number of repeat prescriptions prescribed is to be in accordance with the relevant 'Note' attached to this listing. The approved Product Information recommended dosing is as follows:</p>	Compliance with Written Authority Required procedures
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C18118	<p>(i) 16 days to less than 2 months of age: 0.15 mg/kg (ii) 2 months to less than 2 years of age: 0.20 mg/kg (iii) 2 years of age and older weighing less than 20 kg: 0.25 mg/kg (iv) 2 years of age and older weighing 20 kg or more: 5 mg</p> <p>In this authority application, state which of (i) to (iv) above applies to the patient. Based on (i) to (iv), prescribe up to:</p> <p>1 unit where (i) applies; 2 units where (ii) applies; 3 units where (iii) applies; 3 units where (iv) applies.</p> <p>Pre-symptomatic spinal muscular atrophy (SMA) Initial treatment with this drug of pre-symptomatic spinal muscular atrophy (SMA) Must be treated by a specialist medical practitioner experienced in the diagnosis/management of SMA; OR Must be treated by a medical practitioner who has been directed to prescribe this benefit by a specialist medical practitioner experienced in the diagnosis/management of SMA. The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR The condition must have genetic confirmation of 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; AND The condition must have genetic confirmation that there are 1 to 2 copies of the survival motor neuron 2 (SMN2) gene; AND The condition must be pre-symptomatic; AND The treatment must be given concomitantly with best supportive care for this condition; AND Patient must not have received PBS-subsidised gene therapy. Patient must be aged under 36 months prior to commencing treatment. Application for authorisation of initial treatment must be in writing (lodged via postal service or electronic upload) and must include: (a) details of the proposed prescription; and (b) a completed Spinal muscular atrophy PBS Authority Application Form which includes the following: (i) confirmation of genetic diagnosis of SMA; and (ii) a copy of the results substantiating the number of SMN2 gene copies determined by quantitative polymerase chain reaction (qPCR) or multiple ligation dependent probe amplification (MLPA) The quantity of drug and number of repeat prescriptions prescribed is to be in accordance with</p>	Compliance with Written Authority Required procedures
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the relevant 'Note' attached to this listing.

The approved Product Information recommended dosing is as follows:

(i) 16 days to less than 2 months of age: 0.15 mg/kg

(ii) 2 months to less than 2 years of age: 0.20 mg/kg

(iii) 2 years of age and older weighing less than 20 kg: 0.25 mg/kg

(iv) 2 years of age and older weighing 20 kg or more: 5 mg

In this authority application, state which of (i) to (iv) above applies to the patient. Based on (i) to (iv), prescribe up to:

1 unit where (i) applies;

2 units where (ii) applies;

3 units where (iii) applies;

3 units where (iv) applies.