5.24 RISDIPLAM, Tablet 5 mg Evrysdi®, Roche Products Pty Limited

1 Purpose of Submission

1.1 The Category 4 submission requested a Section 85 General Schedule listing of a new form of risdiplam (risdiplam 5 mg tablet; RIS tablet from herein) (Evrysdi®) under the same circumstances as the currently listed risdiplam 0.75 mg/mL powder for oral liquid, 80 mL (RIS oral liquid from herein) for the treatment of spinal muscular atrophy (SMA) on the Pharmaceutical Benefits Scheme (PBS).

1.2 Listing was requested on a cost-minimisation approach versus the RIS oral liquid.

2 Background

Registration status

2.1 Risdiplam 5 mg tablet blister pack was registered in the Australian Register of Therapeutic Goods (ARTG) on 29 September 2025.

Previous PBAC consideration

2.2 RIS tablet has not been previously considered by the Pharmaceutical Benefits Advisory Committee (PBAC) for the treatment of SMA.

2.3 RIS oral liquid was previously considered and recommended by the PBAC at its March 2021, March 2023 and July 2024 meeting for different patient populations. RIS oral liquid is currently listed under the Section 100 Highly Specialised Drug (HSD) for the treatment of SMA.

3 Requested listing

3.1 The submission requested a Section 85 General Schedule listing of RIS tablet under the same restrictions as the currently listed RIS oral liquid under the Section 100 HSD listing.

3.2 The submission requested the following new RIS tablet listings for patients 2 years of age or older, weighing 20 kg or more. Secretariat suggested additions are in italics and deletions are in strikethrough.

Adult SMA

The requested listings corresponding to the current risdiplam oral solution initial (PBS item codes 13654P and 13632L) and continuing (PBS item codes 13656R and 13646F) restrictions are presented below for the proposed risdiplam 5mg tablet.

13654P 13632L – Adult SMA (Initial)

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13654P HB (S100 HSD Public)	1	3	7	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13632L HS (S100 HSD Private)	1	3	7	Evrysdi
risdiplam 5 mg tablet, 28	*NEW*	*1*	*28*	*0*	*Evrysdi*
Restriction Summary [14391] / Treatment of Concept: [14368]					
Category / Program: ☒ *GENERAL - General Schedule (Code GE)*					
Prescriber type: ☒ Medical Practitioners					
Restriction type: ☒ Authority Required (FULL assessment) in writing only via post/HPOS upload)					
Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday)., Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au, Applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos, Or mailed to:, Services Australia, Complex Drugs, Reply Paid 9826, HOBART TAS 7001					
Administrative Advice: An outcome on the authority application is not immediate, but will follow in due course. Electronic upload is encouraged to reduce processing time.					
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.					
Administrative Advice: No increase in the maximum number of repeats may be authorised.					
Administrative Advice: Special Pricing Arrangements apply.					
Indication: Spinal muscular atrophy (SMA)					
Treatment Phase Initial PBS-subsidised treatment with this drug in an adult who did not initiate PBS subsidy with this drug during childhood					
Clinical criteria:					
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					
Clinical criteria:					
Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug					
Treatment criteria:					
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					
AND					
Treatment criteria:					

Patient must be undergoing initial PBS-subsidised treatment with this drug for untreated disease; or
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)
AND
Treatment criteria:
Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment
Population criteria:
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
Population criteria:
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)
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.
Prescribing Instructions: The authority application must be made in writing and must include:, (1) ~~a completed authority prescription form~~ *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).
Prescribing Instructions: Signs and symptoms of spinal muscular atrophy in the context of this PBS restriction are:, (i) Failure to meet or regression in ability to perform age-appropriate motor milestones, (ii) Proximal weakness, (iii) Hypotonia, (iv) Absence of deep tendon reflexes, (v) Failure to gain weight appropriate for age, (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.
Prescribing Instructions: 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.

MEDICINAL PRODUCT medicinal product pack	**PBS item code**	**Max. qty packs**	**Max. qty units**	**№.of Rpts**	**Available brands**
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13654P HB (S100 HSD Public)	1	3	7	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13632L HS (S100 HSD Private)	1	3	7	Evrysdi
risdiplam 5 mg tablet, 28	NEW	1	28	0	Evrysdi

Restriction Summary [14391] / Treatment of Concept: [14368]
Category / Program: ☒ GENERAL - General Schedule (Code GE)
Prescriber type: ☒ Medical Practitioners
Restriction type: ☒ Authority Required (FULL assessment) in writing only via post/HPOS upload)
Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday)., Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au, Applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos, Or mailed to:, Services Australia, Complex Drugs, Reply Paid 9826, HOBART TAS 7001
Administrative Advice: An outcome on the authority application is not immediate, but will follow in due course. Electronic upload is encouraged to reduce processing time.
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.
Administrative Advice: No increase in the maximum number of repeats may be authorised.

Administrative Advice: Special Pricing Arrangements apply.
Indication: Spinal muscular atrophy (SMA)
Treatment Phase Initial PBS-subsidised treatment with this drug in an adult who did not initiate PBS subsidy with this drug during childhood
Clinical criteria:
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
Clinical criteria:
Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug
Treatment criteria:
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
AND
Treatment criteria:
Patient must be undergoing initial PBS-subsidised treatment with this drug for untreated disease; or
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)
AND
Treatment criteria:
Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment
Population criteria:
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
Population criteria:
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)
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.
Prescribing Instructions: The authority application must be made in writing and must include:, (1) ~~a completed authority prescription form~~ 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).
Prescribing Instructions: Signs and symptoms of spinal muscular atrophy in the context of this PBS restriction are:, (i) Failure to meet or regression in ability to perform age-appropriate motor milestones, (ii) Proximal weakness, (iii) Hypotonia, (iv) Absence of deep tendon reflexes, (v) Failure to gain weight appropriate for age, (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.
Prescribing Instructions: 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.

13646F; 13656R – Adult SMA (continuing/maintenance)

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
RISDIPLAM					

risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13656RHB	3	3	5	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13646FHS	3	3	5	Evrysdi
risdiplam 5 mg tablet, 28	*NEW*	*1*	*28*	*5*	*Evrysdi*

Restriction Summary [14393] / Treatment of Concept: [14420]
Category / Program: ☒ *GENERAL - General Schedule (Code GE)*
Prescriber type: ☒ Medical Practitioners
Restriction type: ☒ Authority Required (immediate assessment) – telephone/online
Administrative Advice: Literature references for various instruments measuring motor function and quality of life in the context of spinal muscular atrophy are:, Revised Upper Limb Module, Mazzone et al. 2017. Revised upper limb module for spinal muscular atrophy: Development of a new module. Muscle & Nerve 55(6):869-874, Hammersmith Functional Motor Scale - Expanded, Ramsey et al. 2017. Revised Hammersmith Scale for spinal muscular atrophy: A SMA specific clinical outcome assessment tool. PLoS ONE 12(2): e0172346. doi:10.1371/journal.pone.0172346., 6-Minute Walk Test (6MWT), American Thoracic Society. 2002. ATS statement: Guidelines for the six-minute walk test. American Journal of Respiratory and Critical Care Medicine 166(1), pp 111-117, The National Hearth Foundation of Australia has 6MWT test standardised instructions and recording forms located at: https://www.heartonline.org.au/resources/documents-and-links#exercise, SMA Health Index, Zizzi et al. 2021. The Spinal Muscular Atrophy Health Index (SMA-HI): A Novel Outcome for Measuring How a Patient Feels and Functions. Muscle & Nerve 63(10), pp 837-844, SMA Functional Rating Scale, Elsheikh et al. 2018. Reliability of Spinal Muscular Atrophy Functional Rating Scale (SMAFRS) in Ambulatory Adults with Spinal Muscular Atrophy. Neurology April (15 Supplement) P4.452
Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.
Administrative Advice: No increase in the maximum number of repeats may be authorised
Administrative Advice: Special Pricing Arrangements apply.
Indication: Spinal muscular atrophy (SMA)
Treatment Phase Continuing/maintenance treatment in an adult where treatment was initiated in adulthood
Clinical criteria:
The treatment must be each of: (i) occurring from week 104 onwards relative to the first administered dose, (ii) demonstrating a clinically meaningful response; or
The treatment must be occurring within the first 104 weeks from the first administered dose
AND
Clinical criteria:
Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug
Treatment criteria:
Patient must be undergoing continuation of existing PBS-subsidised treatment with this drug; or
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
Treatment criteria:
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
AND
Treatment criteria:

Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.
Prescribing Instructions: Where this authority application seeks to continue treatment beyond the first 104 weeks of treatment, comprehensive assessment must be undertaken periodically and documented, involving the patient and the treating physician to establish agreement that treatment is continuing to produce a clinically meaningful response., A clinically meaningful response is present where an improvement, stabilisation or minimal decline in symptoms has occurred as a result of this drug treatment and where there is agreement between the treating physician and patient over what constitutes improvement, stabilisation, or minimal decline., PBS subsidy must cease if there is no agreement on whether a clinically meaningful response is present., Undertake re-assessments for a clinically meaningful response at least every six months. Document these re-assessments in the patient's medical records., In undertaking comprehensive assessments, where practical, a clinically meaningful response assessment encompasses the patient's motor function as assessed using an instrument like the Revised Upper Limb Module (RULM), Hammersmith Functional Motor Scale - Expanded (HFMSE) or 6-minute walk test (6MWT), and the patient's quality of life including, but not limited to, level of independence. Quality of life may be informed by use of the SMA Health Index (SMA-HI) or SMA Functional Rating Scale (SMAFRS).

Paediatric Symptomatic Type I, II or IIIa SMA

The requested listings corresponding to the current risdiplam oral solution initial (PBS item codes 12614X and 12610Q) and continuing (PBS item codes 12606L and 12609P) restrictions are presented below for the risdiplam 5mg tablet. The sponsor has proposed a population criterion for the qualifying age and weight for the requested population.

12610Q; 12614X – Paediatric Symptomatic Type I, II or IIIa SMA (Initial)

MEDICINAL PRODUCT medicinal product pack	**PBS item code**	**Max. qty packs**	**Max. qty units**	**№.of Rpts**	**Available brands**
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	12610Q HS	1	1	0	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	12614X HB	1	1	0	Evrysdi
risdiplam 5 mg tablet, 28	*NEW*	*1*	*28*	*0*	*Evrysdi*
Restriction Summary [14371]/ Treatment of Concept: [14372]					
Category / Program: ☒ *GENERAL - General Schedule (Code GE)*					
Prescriber type: ☒ Medical Practitioners					
Restriction type: ☒ Authority Required (FULL assessment) in writing only via post/HPOS upload)					
Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday)., Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au, Applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos, Or mailed to:, Services Australia, Complex Drugs, Reply Paid 9826, HOBART TAS 7001					
Administrative Advice: No increase in the maximum number of repeats may be authorised.					
Administrative Advice: Special Pricing Arrangements apply.					
Indication: Symptomatic Type I, II or IIIa spinal muscular atrophy (SMA)					
Treatment Phase Initial treatment					
Clinical criteria:					

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
Clinical criteria:
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
Clinical criteria:
The treatment must be given concomitantly with best supportive care for this condition
AND
Clinical criteria:
The treatment must not be in combination with PBS-subsidised treatment with nusinersen for this condition
AND
Clinical criteria:
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
AND
Clinical criteria:
Patient must be untreated with gene therapy
Treatment criteria:
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
Population criteria:
~~Patient must be 18 years of age or under~~
Patient must be between 2 and 18 years of age and weigh 20 kg or more
Prescribing Instructions: 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.
Prescribing Instructions: 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.
Prescribing Instructions: 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.
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.
Prescribing Instructions: Application for authorisation of initial treatment must be in writing and must include:, (a) *details of the proposed prescription* ~~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).
Prescribing Instructions: 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

~~Prescribing Instructions: 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.~~

12606L; 12609P - Symptomatic Type I, II, IIIa /Pre-symptomatic SMA (1 or 2 SMN2 gene copies) – (Continuing/maintenance)

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	12606L HB	1	1	5	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	12609P HS	1	1	5	Evrysdi
risdiplam 5 mg tablet, 28	*NEW*	*1*	*28*	*5*	*Evrysdi*
Restriction Summary [15113] / Treatment of Concept: [15095]					
Category / Program: ☒ *GENERAL - General Schedule (Code GE)*					
Prescriber type: ☒ Medical Practitioners					
Restriction type: ☒ Authority Required (immediate assessment) – telephone/online					
Administrative Advice: ~~The maximum quantity of drug to be subsidised per dispensing, as well as the number of repeat prescriptions is to be as follows:, Patient weight greater than 19 kg: up to 3 units per dispensing, with up to 5 repeat prescriptions, Patient weight between 17 kg to 19 kg: up to 3 units per dispensing, with up to 4 repeat prescriptions, Patient weight between 13 kg to 17 kg: up to 2 units per dispensing, with up to 5 repeat prescriptions, Patient weight between 10 kg up to 13 kg: up to 2 units per dispensing, with up to 4 repeat prescriptions, Patient weight less than 10 kg: up to 1 unit per dispensing, with up to 5 repeat prescriptions~~					
Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).					
Administrative Advice: No increase in the maximum number of repeats may be authorised.					
Administrative Advice: Special Pricing Arrangements apply.					
Indication: Spinal muscular atrophy (SMA)					
Treatment Phase Continuing/maintenance treatment with this drug of either symptomatic Type I, II or IIIa SMA, or, pre-symptomatic SMA (1 or 2 copies of the SMN2 gene)					
Clinical criteria:					
Patient must have previously received PBS-subsidised treatment with this drug for this condition; or					
Patient must be eligible for continuing PBS-subsidised treatment with nusinersen for this condition					
AND					
Clinical criteria:					
The treatment must not be in combination with PBS-subsidised treatment with nusinersen for this condition					
AND					
Clinical criteria:					
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					
AND					
Clinical criteria:					
The treatment must be given concomitantly with best supportive care for this condition					
Treatment criteria:					
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					

AND
Treatment criteria:
Patient must not be undergoing treatment through this 'Continuing treatment' listing where the most recent PBS authority approval for this PBS indication has been for gene therapy
Population criteria
Patient must have been 18 years of age or younger at the time of initial treatment with this drug;
AND
Population criteria
Patient must be 2 years of age or older, weigh 20 kg or more
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.
Prescribing Instructions: In a patient who wishes to switch from PBS-subsidised nusinersen to PBS-subsidised risdiplam for this condition a wash out period may be required.
~~Prescribing Instructions: The quantity of drug and number of repeat prescriptions prescribed is to be in accordance with the relevant 'Note' attached to this listing.~~
Prescribing Instructions: 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
~~Prescribing Instructions: 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.~~

Paediatric Pre-symptomatic SMA with 3 SMN2 gene copies

The requested listings corresponding to the current risdiplam oral solution continuing (PBS item codes 14646W/14639L) restrictions are presented below for the risdiplam 5mg tablet. The sponsor has proposed a population criterion for the qualifying age and weight for the requested population.

14639L; 14646W - Pre-symptomatic SMA (3 copies SMN2 gene) – Continuing/Maintenance

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	14639L HS	1	1	5	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	14646W HB	1	1	5	Evrysdi
risdiplam 5 mg tablet, 28	*NEW*	*1*	*28*	*5*	*Evrysdi*
Restriction Summary [16044] / Treatment of Concept: [15986]					
Category / Program: ☒ *GENERAL - General Schedule (Code GE)*					
Prescriber type: ☒ Medical Practitioners					
Restriction type: ☒ Authority Required (immediate assessment) – telephone/online					
Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).					
Administrative Advice: No increase in the maximum number of repeats may be authorised.					
Administrative Advice: Special Pricing Arrangements apply.					
Indication: Pre-symptomatic spinal muscular atrophy (SMA)					

Treatment Phase Continuing/maintenance treatment of pre-symptomatic spinal muscular atrophy (SMA) with 3 copies of the SMN2 gene					
Clinical criteria:					
Patient must have previously received PBS-subsidised treatment with this drug for this condition; or					
Patient must be eligible for continuing PBS-subsidised treatment with nusinersen for this condition					
AND					
Clinical criteria:					
The treatment must not be in combination with PBS-subsidised treatment with nusinersen for this condition					
AND					
Clinical criteria:					
The treatment must be given concomitantly with best supportive care for this condition					
AND					
Clinical criteria:					
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					
Treatment criteria:					
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					
AND					
Treatment criteria:					
Patient must not be undergoing treatment through this 'Continuing treatment' listing where the most recent PBS authority approval for this PBS indication has been for gene therapy					
Population criteria					
Patient must have been 18 years of age or younger at the time of initial treatment with this drug					
	AND				
	Population criteria				
Patient must be 2 years of age or older, weigh 20 kg or more					
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.					
Prescribing Instructions: In a patient who wishes to switch from PBS-subsidised nusinersen to PBS-subsidised risdiplam for this condition a wash out period may be required.					
~~Prescribing Instructions: The quantity of drug and number of repeat prescriptions prescribed is to be in accordance with the relevant 'Note' attached to this listing.~~					
Prescribing Instructions: 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					
~~Prescribing Instructions: 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.~~					
MEDICINAL PRODUCT medicinal product pack	**PBS item code**	**Max. qty packs**	**Max. qty units**	**№.of Rpts**	**Available brands**
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	14639L HS	1	1	5	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	14646W HB	1	1	5	Evrysdi
risdiplam 5 mg tablet, 28	NEW	1	28	5	Evrysdi
Restriction Summary [16044] / Treatment of Concept: [15986]					

Category / Program: ☒ GENERAL - General Schedule (Code GE)
Prescriber type: ☒ Medical Practitioners
Restriction type: ☒ Authority Required (immediate assessment) – telephone/online
Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).
Administrative Advice: No increase in the maximum number of repeats may be authorised.
Administrative Advice: Special Pricing Arrangements apply.
Indication: Pre-symptomatic spinal muscular atrophy (SMA)
Treatment Phase Continuing/maintenance treatment of pre-symptomatic spinal muscular atrophy (SMA) with 3 copies of the SMN2 gene
Clinical criteria:
Patient must have previously received PBS-subsidised treatment with this drug for this condition; or
Patient must be eligible for continuing PBS-subsidised treatment with nusinersen for this condition
AND
Clinical criteria:
The treatment must not be in combination with PBS-subsidised treatment with nusinersen for this condition
AND
Clinical criteria:
The treatment must be given concomitantly with best supportive care for this condition
AND
Clinical criteria:
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
Treatment criteria:
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
AND
Treatment criteria:
Patient must not be undergoing treatment through this 'Continuing treatment' listing where the most recent PBS authority approval for this PBS indication has been for gene therapy
Population criteria
Patient must have been 18 years of age or younger at the time of initial treatment with this drug
AND
Population criteria
Patient must be 2 years of age or older, weigh 20 kg or more
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.
Prescribing Instructions: In a patient who wishes to switch from PBS-subsidised nusinersen to PBS-subsidised risdiplam for this condition a wash out period may be required.
~~Prescribing Instructions: The quantity of drug and number of repeat prescriptions prescribed is to be in accordance with the relevant 'Note' attached to this listing.~~
Prescribing Instructions: 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
~~Prescribing Instructions: 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.~~

Paediatric Symptomatic type IIIB/IIIC SMA

The requested listings corresponding to the current risdiplam oral solution initial (PBS item codes 13633M and 13639W) and continuing (PBS item codes 13631K and 13659X) restrictions are presented below for the risdiplam 5mg tablet. The sponsor has proposed a population criterion for the qualifying age and weight for the requested population.

13633M; 13639W – Paediatric Symptomatic type IIIB/IIIC SMA (Initial)

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13633M HB	3	3	5	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13639W HS	3	3	5	Evrysdi
risdiplam 5 mg tablet, 28	*NEW*	*1*	*28*	*0*	*Evrysdi*
Restriction Summary [14395] / Treatment of Concept: [14408]					
Category / Program: ☒ *GENERAL - General Schedule (Code GE)*					
Prescriber type: ☒ Medical Practitioners					
Restriction type: ☒ Authority Required (FULL assessment) in writing only via post/HPOS upload)					
Administrative Advice: ~~The maximum quantity of drug to be subsidised per dispensing, as well as the number of repeat prescriptions is to be as follows:, Patient weight greater than 19 kg: up to 3 units per dispensing, with up to 5 repeat prescriptions, Patient weight between 17 kg to 19 kg: up to 3 units per dispensing, with up to 4 repeat prescriptions, Patient weight between 13 kg to 17 kg: up to 2 units per dispensing, with up to 5 repeat prescriptions, Patient weight between 10 kg up to 13 kg: up to 2 units per dispensing, with up to 4 repeat prescriptions, Patient weight less than 10 kg: up to 1 unit per dispensing, with up to 5 repeat prescriptions~~					
Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday)., Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au, Applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos, Or mailed to:, Services Australia, Complex Drugs, Reply Paid 9826, HOBART TAS 7001					
Administrative Advice: An outcome on the authority application is not immediate, but will follow in due course. Electronic upload is encouraged to reduce processing time.					
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.					
Administrative Advice: No increase in the maximum number of repeats may be authorised.					
Administrative Advice: Special Pricing Arrangements apply.					
Indication: Symptomatic type IIIB/IIIC spinal muscular atrophy (SMA)					
Treatment Phase Initial PBS-subsidised treatment with this drug in a child					
Population criteria:					
Patient must be of an age that is prior to their 19th birthday at the time of this authority application;					
AND					
Population criteria:					
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);					
AND					
Population criteria:					
Patient must be 2 years of age or older, weigh 20 kg or more					
Clinical criteria:					
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					
Clinical criteria:					

Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug
Treatment criteria:
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
AND
Treatment criteria:
Patient must be undergoing initial PBS-subsidised treatment with this drug for untreated disease; or
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)
AND
Treatment criteria:
Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.
Prescribing Instructions: The authority application must be made in writing and must include:, (1) *details of the proposed prescription* ~~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).
Prescribing Instructions: Signs and symptoms of spinal muscular atrophy in the context of this PBS restriction are:, (i) Failure to meet or regression in ability to perform age-appropriate motor milestones, (ii) Proximal weakness, (iii) Hypotonia, (iv) Absence of deep tendon reflexes, (v) Any active denervation or chronic neurogenic changes found on electromyography, (vi) A compound muscle action potential below normative values for an age-matched child.
Prescribing Instructions: In this authority application, confirm:, (1) the patient's medical history is consistent with a diagnosis of type IIIB/IIIC spinal muscular atrophy, (2) which of the above (i to vi) (at least 1) were present after their 3rd birthday, but before their 19th birthday, (3) the age of the patient (rounded to the nearest year) when the first sign/symptom was observed.
~~Prescribing Instructions: The quantity of drug and number of repeat prescriptions prescribed is to be in accordance with the relevant 'Note' attached to this listing.Sof~~
Prescribing Instructions: 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
~~Prescribing Instructions: 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.~~

13631K 13659X - Paediatric/Adult Symptomatic type IIIB/IIIC SMA (Continuing/maintenance)

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13631K HB	3	3	5	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13659X HS	3	3	5	Evrysdi
risdiplam 5 mg tablet, 28	*NEW*	*1*	*28*	*5*	*Evrysdi*
Restriction Summary [14394] / Treatment of Concept: [14392]					
Category / Program: ☒ *GENERAL - General Schedule (Code GE)*					
Prescriber type: ☒ Medical Practitioners					
Restriction type: ☒ Authority Required (immediate assessment) – telephone/online					
Administrative Advice: ~~The maximum quantity of drug to be subsidised per dispensing, as well as the number of repeat prescriptions is to be as follows:, Patient weight greater than 19 kg: up to 3 units per dispensing, with up to 5 repeat prescriptions, Patient weight between 17 kg to 19 kg: up to 3 units per dispensing, with up to 4 repeat prescriptions, Patient weight between 13 kg to 17 kg: up to 2 units per dispensing, with up to 5 repeat prescriptions, Patient weight between 10 kg up to 13 kg: up to 2 units per dispensing, with up to 4 repeat prescriptions, Patient weight less than 10 kg: up to 1 unit per dispensing, with up to 5 repeat prescriptions~~					
Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).					
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.					
Administrative Advice: No increase in the maximum number of repeats may be authorised.					
Administrative Advice: Special Pricing Arrangements apply.					
Indication: Symptomatic type IIIB/IIIC spinal muscular atrophy (SMA)					
Treatment Phase Continuing/maintenance treatment in a child or adult, but where treatment was initiated during childhood					
Clinical criteria:					
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					
Treatment criteria:					
Patient must be undergoing continuation of existing PBS-subsidised treatment with this drug; or					
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					
Treatment criteria:					
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					
AND					
Treatment criteria:					
Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment					
Population criteria:					
Patient must be 2 years of age or older, weigh 20 kg or more					
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.					

~~Prescribing Instructions: The quantity of drug and number of repeat prescriptions prescribed is to be in accordance with the relevant 'Note' attached to this listing.~~					
Prescribing Instructions: 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					
~~Prescribing Instructions: 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.~~					
MEDICINAL PRODUCT medicinal product pack	**PBS item code**	**Max. qty packs**	**Max. qty units**	**№.of Rpts**	**Available brands**
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13631KHB	3	3	5	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13659XHS	3	3	5	Evrysdi
risdiplam 5 mg tablet, 28	*NEW*	*1*	*28*	*5*	*Evrysdi*
Restriction Summary [14394] / Treatment of Concept: [14392]					
Category / Program: ☒ GENERAL - General Schedule (Code GE)					
Prescriber type: ☒ Medical Practitioners					
Restriction type: ☒ Authority Required (immediate assessment) – telephone/online					
~~**Administrative Advice:** The maximum quantity of drug to be subsidised per dispensing, as well as the number of repeat prescriptions is to be as follows:, Patient weight greater than 19 kg: up to 3 units per dispensing, with up to 5 repeat prescriptions, Patient weight between 17 kg to 19 kg: up to 3 units per dispensing, with up to 4 repeat prescriptions, Patient weight between 13 kg to 17 kg: up to 2 units per dispensing, with up to 5 repeat prescriptions, Patient weight between 10 kg up to 13 kg: up to 2 units per dispensing, with up to 4 repeat prescriptions, Patient weight less than 10 kg: up to 1 unit per dispensing, with up to 5 repeat prescriptions~~					
Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).					
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.					
Administrative Advice: No increase in the maximum number of repeats may be authorised.					
Administrative Advice: Special Pricing Arrangements apply.					
Indication: Symptomatic type IIIB/IIIC spinal muscular atrophy (SMA)					
Treatment Phase Continuing/maintenance treatment in a child or adult, but where treatment was initiated during childhood					
Clinical criteria:					
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					
Treatment criteria:					
Patient must be undergoing continuation of existing PBS-subsidised treatment with this drug; or					
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					
Treatment criteria:					
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					
AND					
Treatment criteria:					

Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment
Population criteria:
Patient must be 2 years of age or older, weigh 20 kg or more
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.
~~Prescribing Instructions: The quantity of drug and number of repeat prescriptions prescribed is to be in accordance with the relevant 'Note' attached to this listing.~~
Prescribing Instructions: 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
~~Prescribing Instructions: 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.~~

These restrictions may be subject to further review. Should there be any changes made to the restriction the sponsor will be informed.

3.3 The submission claimed that the proposed listing of RIS tablet would offer the following benefits as compared to the RIS oral liquid:

i) Enhanced patient convenience: The availability of RIS tablet simplifies daily administration, reducing treatment burden on patients and caregivers by eliminating the need for preparation and pipetting, and allowing easy tracking of doses;

ii) Potential for greater independence and freedom: The availability of RIS tablet allows patient to swallow whole or dispersed in water as a suspension, does not require refrigeration and would be available for access at community pharmacies. This allows patients to integrate into daily activities such as work, travel and education; and

iii) Lower resource utilisation where health care professionals would not need to prepare treatment and therefore a reduction in the use of consumables associated with its preparation and administration relative to the RIS oral liquid.

3.4 The HSD Program contains medicines which, because of their clinical use and other special features, have restrictions on where they can be prescribed and supplied. In most cases, medical practitioners are required to undertake specific training or be affiliated with a specialised hospital unit to prescribe HSDs. Medicines listed on the General Schedule do not commonly share these same requirements.

3.5 At the March 2021 PBAC meeting, a Section 100 HSD listing was requested for RIS oral liquid to provide flexibility for the treatment to be dispensed in either a hospital or community pharmacy setting given the medicine can be taken orally at home (risdiplam main submission body, March 2021). The RIS oral liquid has since been recommended and listed under the Section 100 HSD Public or HSD Private prescription for the initial and continuing treatment of patients receiving treatment in, at or from a hospital.

3.6 The sponsor proposed a listing requesting the same treatment criteria to the currently listed RIS oral liquid under the Section 85 General Schedule, in that for both initiation and continuation of therapy, non-specialist medical practitioners must prescribe in consultation with (or as directed by) a specialist experienced in the diagnosis and management of SMA. However, a General Schedule listing would enable medical practitioners who are not specialists affiliated with a hospital to prescribe the proposed treatment for both initiation and continuation of therapy, including for patients receiving treatment outside a hospital setting (noting that this may not be a common scenario for patients with SMA).

3.7 Some HSDs are also restricted by the settings in which they can be supplied. For example, HSD non-CAR medicines supplied via a public hospital prescription cannot be dispensed by a s90 community pharmacy whereas those supplied via a private hospital prescription can. The currently listed risdiplam is a Complex Authority Required (CAR) medicine where medicines can be dispensed by a s90 community pharmacy even if supplied on a public hospital prescription. The submission stated that the proposed listing under the General Schedule would expand access through s90 community pharmacies as opposed to outpatient pharmacies and clinics. However, the currently listed form of risdiplam is already accessible through s90 community pharmacies due to its Complex Authority Required (CAR) status.

4 Comparator

4.1 The submission nominated RIS oral liquid as the main comparator. This is appropriate.

5 Consideration of the evidence

Sponsor hearing

5.1 There was no hearing for this item.

Consumer comments

5.2 The PBAC noted and welcomed input from an individual (1), parent (1) and the National Paediatric Medicines Forum. Their comments described how a tablet formulation of risdiplam would provide some patients independence by simplifying dosing, and how travelling with tablets would be more practical than transporting a refrigerated glass bottle. All consumers noted that the size of the tablet would be an important determining factor in whether the patient would be able to swallow it, and that having both the oral solution and tablet formulations available would be necessary for equitable access.

Clinical trials

5.3 The submission's request was based on study BP42066, a phase I clinical study, conducted to compare the bioavailability and bioequivalence between the two drug formulations (5 mg RIS tablet and 5 mg RIS oral liquid).

5.4 The submission stated that pharmacokinetics evidence from the study supported the bioequivalence of RIS tablet and RIS oral liquid. Details of the study are summarised in Table 1 below.

Table 1: Trial presented in the submission

Trial ID	Protocol title/ Publication title	Publication citation
NCT04718181	Study BP42066 Bioavailability and Bioequivalence of Two Risdiplam Tablets in Healthy Participants Hugh A. Coleman, Risdiplam – A Phase I, Open-label, Multiperiod Crossover Study to Investigate the Safety, Food Effect, Bioavailability, and Bioequivalence of Oral Doses of Two Different Formulations in Healthy Subjects	13 March 2023

Source: '2.1.1 Study BP42066 design' (p14) main submission

5.5 As a Category 4 submission, the clinical evaluation has not been independently evaluated.

Clinical claim

5.6 The submission claimed RIS tablet is non-inferior to RIS oral liquid in terms of effectiveness and safety. The Therapeutic Goods Administration's Delegate's Overview supported that the RIS tablet is bioequivalent to the RIS oral liquid.

5.7 The PBAC considered that the claim of non-inferior effectiveness was reasonable.

5.8 The PBAC considered that the claim of non-inferior safety was reasonable.

Economic analysis

5.9 As a Category 4 submission, the economic analysis has not been independently evaluated.

5.10 A cost-minimisation approach has been requested for the listing of RIS tablet compared with RIS oral liquid.

5.11 The submission proposed the equi-effective doses to be RIS 5 mg tablet once daily = RIS 5 mg oral liquid once daily. The calculations are provided in Table 2 below.

Table 2: Cost-minimisation analysis of RIS oral liquid vs RIS tablet

Dosage	Risdiplam 0.75 mg/mL powder for oral liquid, 80 mL	Risdiplam 5 mg tablet
Strength (A)	60 mg	5 mg
Quantity (B)	1 bottle	28 tablets
Dose per day (C)	5 mg	5 mg
Doses per bottle/ tablet pack (D)	(A/C) = 12	28
Doses per year (E)	365.25	365.25
Bottles/ tablet packs per year (F)	(E/D) = 30.44	(E/D) = 13.04
Maximum quantity units per script (G)	3.00	1.00
Scripts per year (H)	(F/G) = 10.15	(F/G) = 13.04
AEMP per bottle/ tablet pack (J)	$10.841.89	[($10,841.89*30.44)/13.04] = $25,297.74

Source: Cost-minimisation analysis Evrysdi (risdiplam) tablet workbook

5.12 The proposed published and effective ex-manufacturer prices for RIS tablet are presented in Table 3 below. Similar to the existing PBS listings of RIS oral liquid, a Special Pricing Arrangement (SPA) was requested for the proposed listing.

Table 3: Existing and proposed prices of risdiplam

	Risdiplam 0.75 mg/mL powder for oral liquid, 80mL	Risdiplam 5 mg tablet
Published prices for all requested SMA populations		
AEMP per bottle / tablet pack ($)	$10,841.89	$25,297.74
Effective prices for each requested SMA population		
Adult SMA		
EEMP per bottle / tablet pack ($)	[redacted]	[redacted]
Symptomatic Type 1, 2 or 3a children		
EEMP per bottle / tablet pack ($)	[redacted]	[redacted]
Symptomatic Type 3b or 3c children		
EEMP per bottle / tablet pack ($)	[redacted]	[redacted]
Pre-symptomatic newborns with 3 SMN2 gene copies		
EEMP per bottle / tablet pack ($)	[redacted]	[redacted]

Source: Main submission body

Estimated PBS usage and financial implications

5.13 The submission estimated the utilisation and costs of the proposed listing based on market-share approach.

5.14 Table 4 presented the estimated extent of use and the net financial implications to the PBS. No impact on the RPBS is reported as no patients are currently accessing risdiplam through the RPBS. The financial impact to Services Australia will be determined by that agency as part of the post PBAC process.

5.15 The submission estimated that the proposed listing would result in a net financial impact of $0 to < $10 million to the PBS over the next six years (see Table 4 below).

5.16 The submission stated that the net cost to the Commonwealth stemmed from the higher fees and mark-ups related to the proposed listing under the Section 85 General Schedule compared to the existing listing under the Section 100 HSD.

Table 4: Estimated use and financial implications on Section 85 General Schedule (based on effective price)

	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6
Estimated extent of use						
Number of scripts dispensed[a]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]
Estimated financial implications of risdiplam 5mg tablets						
Cost to PBS/RPBS less co-payment ($)	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]
Net financial implications						
Net cost to PBS/RPBS ($)	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]	[redacted][2]

[a] Assuming 13.04 scripts per patient per year as estimated by the submission.
Abbreviations: PBS = Pharmaceutical Benefits Scheme; RPBS = Repatriation Pharmaceutical Benefits Scheme.
Source: Table 4.3, page 28 of the main submission
The redacted values correspond to the following ranges:
[1] 500 to < 5,000
[2] $0 to < $10 million

5.17 If proposed listing is listed under Section 100 HSD, there would be an estimated save of $0 to < $10 million the Commonwealth (see Table 5 below) due to impacts on fees and markups in respective programs, including the number of copayments.

Table 5: Estimated use and financial implications on Section 100 HSD (Public/Private) (based on effective price)

	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6
Estimated extent of use						
Number of scripts dispensed[a]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]
Estimated financial implications of risdiplam 5mg tablets						
Cost to PBS/RPBS less co-payment	-$[redacted][2]	-$[redacted][2]	-$[redacted][2]	-$[redacted][2]	-$[redacted][2]	-$[redacted][2]
Net financial implications						
Net cost to PBS/RPBS	-$[redacted][2]	-$[redacted][2]	-$[redacted][2]	-$[redacted][2]	-$[redacted][2]	-$[redacted][2]

The redacted values correspond to the following ranges:
[1] 500 to < 5,000
[2] $0 to < $10 million

Financial Management - Risk Sharing Arrangements

5.18 There are existing risk sharing arrangements (RSAs) in place for the current PBS listing of RIS oral liquid.

6 PBAC Outcome

6.1 The PBAC recommended the listing of risdiplam 5 mg tablets (Evrysdi) for the treatment of spinal muscular atrophy (SMA), on the basis that it should be available only under special arrangements as a complex authority required (CAR) medicine. The PBAC noted the sponsor's request for a Section 85 General Schedule listing, however,

recommended a Section 100 HSD listing to maintain consistency with the oral solution form.

6.2 The PBAC is satisfied that risdiplam tablets, for some patients, offer a more convenient alternative to the oral solution, due to their transportability and dispersibility properties.

6.3 The PBAC accepted the claims of non-inferior clinical effectiveness and safety of risdiplam tablet compared with risdiplam oral solution.

6.4 The PBAC advised that the estimated financial impact is reasonable. The PBAC recommend that the RIS tablet be included in the same RSAs as the existing listings for RIS oral liquid.

6.5 The PBAC considered the utilisation estimates to be reasonable.

6.6 The PBAC noted that its recommendation was on a cost-minimisation basis and advised that, because risdiplam 5 mg tablet is not expected to provide a substantial and clinically relevant improvement in efficacy, or reduction of toxicity, over risdiplam oral solution or not expected to address a high and urgent unmet clinical need given the presence of an alternative therapy, the criteria prescribed by the *National Health (Pharmaceuticals and Vaccines – Cost Recovery) Regulations 2022* for Pricing Pathway A were not met.

Outcome:
Recommended

7 Recommended Listing

Adult SMA

13654P 13632L – Adult SMA (Initial)

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13654P HB (S100 HSD Public)	1	3	7	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13632L HS (S100 HSD Private)	1	3	7	Evrysdi
risdiplam 5 mg tablet, 28	NEW (Private) NEW (Public)	1	28	5	Evrysdi
Restriction Summary [14391] / Treatment of Concept: [14368]					
Category / Program: ☒ Section 100 – Highly Specialised Drugs Program – Public (Code HB) / Private (Code HS)					
Prescriber type: ☒ Medical Practitioners					
Restriction type: ☒ Authority Required (FULL assessment) in writing only via post/HPOS upload)					
Authority type: ☒ Complex Authority Required (CAR)					

Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday)., Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au, Applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos, Or mailed to:, Services Australia, Complex Drugs, Reply Paid 9826, HOBART TAS 7001
Administrative Advice: An outcome on the authority application is not immediate, but will follow in due course. Electronic upload is encouraged to reduce processing time.
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.
Administrative Advice: No increase in the maximum number of repeats may be authorised.
Administrative Advice: Special Pricing Arrangements apply.
Indication: Spinal muscular atrophy (SMA)
Treatment Phase Initial PBS-subsidised treatment with this drug in an adult who did not initiate PBS subsidy with this drug during childhood
Clinical criteria:
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
Clinical criteria:
Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug
Treatment criteria:
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
AND
Treatment criteria:
Patient must be undergoing initial PBS-subsidised treatment with this drug for untreated disease; or
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)
AND
Treatment criteria:
Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment
Population criteria:
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
Population criteria:
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)
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.
Prescribing Instructions: The authority application must be made in writing and 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).

Prescribing Instructions: Signs and symptoms of spinal muscular atrophy in the context of this PBS restriction are:, (i) Failure to meet or regression in ability to perform age-appropriate motor milestones, (ii) Proximal weakness, (iii) Hypotonia, (iv) Absence of deep tendon reflexes, (v) Failure to gain weight appropriate for age, (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.
Prescribing Instructions: 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.

13646F; 13656R – Adult SMA (continuing/maintenance)

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13656R HB	3	3	5	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13646F HS	3	3	5	Evrysdi
risdiplam 5 mg tablet, 28	NEW (Private) NEW (Public)	1	28	5	Evrysdi

Restriction Summary [14393] / Treatment of Concept: [14420]
Category / Program: ☒ Section 100 – Highly Specialised Drugs Program – Public (Code HB) / Private (Code HS)
Prescriber type: ☒ Medical Practitioners
Restriction type: ☒ Authority Required (immediate assessment) – telephone/online
Authority type: ☒ Complex Authority Required (CAR)
Administrative Advice: Literature references for various instruments measuring motor function and quality of life in the context of spinal muscular atrophy are:, Revised Upper Limb Module, Mazzone et al. 2017. Revised upper limb module for spinal muscular atrophy: Development of a new module. Muscle & Nerve 55(6):869-874, Hammersmith Functional Motor Scale - Expanded, Ramsey et al. 2017. Revised Hammersmith Scale for spinal muscular atrophy: A SMA specific clinical outcome assessment tool. PLoS ONE 12(2): e0172346. doi:10.1371/journal.pone.0172346., 6-Minute Walk Test (6MWT), American Thoracic Society. 2002. ATS statement: Guidelines for the six-minute walk test. American Journal of Respiratory and Critical Care Medicine 166(1), pp 111-117, The National Hearth Foundation of Australia has 6MWT test standardised instructions and recording forms located at: https://www.heartonline.org.au/resources/documents-and-links#exercise, SMA Health Index, Zizzi et al. 2021. The Spinal Muscular Atrophy Health Index (SMA-HI): A Novel Outcome for Measuring How a Patient Feels and Functions. Muscle & Nerve 63(10), pp 837-844, SMA Functional Rating Scale, Elsheikh et al. 2018. Reliability of Spinal Muscular Atrophy Functional Rating Scale (SMAFRS) in Ambulatory Adults with Spinal Muscular Atrophy. Neurology April (15 Supplement) P4.452
Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.
Administrative Advice: No increase in the maximum number of repeats may be authorised
Administrative Advice: Special Pricing Arrangements apply.
Indication: Spinal muscular atrophy (SMA)
Treatment Phase Continuing/maintenance treatment in an adult where treatment was initiated in adulthood
Clinical criteria:
The treatment must be each of: (i) occurring from week 104 onwards relative to the first administered dose, (ii) demonstrating a clinically meaningful response; or
The treatment must be occurring within the first 104 weeks from the first administered dose
AND
Clinical criteria:

Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug
Treatment criteria:
Patient must be undergoing continuation of existing PBS-subsidised treatment with this drug; or
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
Treatment criteria:
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
AND
Treatment criteria:
Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.
Prescribing Instructions: Where this authority application seeks to continue treatment beyond the first 104 weeks of treatment, comprehensive assessment must be undertaken periodically and documented, involving the patient and the treating physician to establish agreement that treatment is continuing to produce a clinically meaningful response., A clinically meaningful response is present where an improvement, stabilisation or minimal decline in symptoms has occurred as a result of this drug treatment and where there is agreement between the treating physician and patient over what constitutes improvement, stabilisation, or minimal decline., PBS subsidy must cease if there is no agreement on whether a clinically meaningful response is present., Undertake re-assessments for a clinically meaningful response at least every six months. Document these re-assessments in the patient's medical records., In undertaking comprehensive assessments, where practical, a clinically meaningful response assessment encompasses the patient's motor function as assessed using an instrument like the Revised Upper Limb Module (RULM), Hammersmith Functional Motor Scale - Expanded (HFMSE) or 6-minute walk test (6MWT), and the patient's quality of life including, but not limited to, level of independence. Quality of life may be informed by use of the SMA Health Index (SMA-HI) or SMA Functional Rating Scale (SMAFRS).

Paediatric Symptomatic Type I, II or IIIa SMA

12610Q; 12614X – Paediatric Symptomatic Type I, II or IIIa SMA (Initial)

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	12610Q HS	1	1	0	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	12614X HB	1	1	0	Evrysdi
risdiplam 5 mg tablet, 28	NEW (Private) NEW (Public)	1	28	5	Evrysdi
Restriction Summary [14371]/ Treatment of Concept: [14372]					
Category / Program: ☒ Section 100 – Highly Specialised Drugs Program – Public (Code HB) / Private (Code HS)					
Prescriber type: ☒ Medical Practitioners					
Restriction type: ☒ Authority Required (FULL assessment) in writing only via post/HPOS upload)					
Authority type: ☒ Complex Authority Required (CAR)					

Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday)., Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au, Applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos, Or mailed to:, Services Australia, Complex Drugs, Reply Paid 9826, HOBART TAS 7001
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.
Administrative Advice: No increase in the maximum number of repeats may be authorised.
Administrative Advice: Special Pricing Arrangements apply.
Indication: Symptomatic Type I, II or IIIa spinal muscular atrophy (SMA)
Treatment Phase Initial treatment
Clinical criteria:
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
Clinical criteria:
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
Clinical criteria:
The treatment must be given concomitantly with best supportive care for this condition
AND
Clinical criteria:
The treatment must not be in combination with PBS-subsidised treatment with nusinersen for this condition
AND
Clinical criteria:
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
AND
Clinical criteria:
Patient must be untreated with gene therapy
Treatment criteria:
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
Population criteria:
Patient must be between 2 and 18 years of age and weigh 20 kg or more
Prescribing Instructions: 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.
Prescribing Instructions: 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.

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

12606L; 12609P - Symptomatic Type I, II, IIIa /Pre-symptomatic SMA (1 or 2 SMN2 gene copies) – (Continuing/maintenance)

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	12606L HB	1	1	5	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	12609P HS	1	1	5	Evrysdi
risdiplam 5 mg tablet, 28	NEW (Private) NEW (Public)	1	28	5	Evrysdi

Restriction Summary [15113] / Treatment of Concept: [15095]
Category / Program: Section 100 – Highly Specialised Drugs Program – Public (Code HB) / Private (Code HS)
Prescriber type: ☒ Medical Practitioners
Restriction type: ☒ Authority Required (immediate assessment) – telephone/online
Authority type: ☒ Complex Authority Required (CAR)
Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.
Administrative Advice: No increase in the maximum number of repeats may be authorised.
Administrative Advice: Special Pricing Arrangements apply.
Indication: Spinal muscular atrophy (SMA)
Treatment Phase Continuing/maintenance treatment with this drug of either symptomatic Type I, II or IIIa SMA, or, pre-symptomatic SMA (1 or 2 copies of the SMN2 gene)
Clinical criteria:
Patient must have previously received PBS-subsidised treatment with this drug for this condition; or
Patient must be eligible for continuing PBS-subsidised treatment with nusinersen for this condition
AND
Clinical criteria:
The treatment must not be in combination with PBS-subsidised treatment with nusinersen for this condition
AND
Clinical criteria:
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
AND
Clinical criteria:

The treatment must be given concomitantly with best supportive care for this condition
Treatment criteria:
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
AND
Treatment criteria:
Patient must not be undergoing treatment through this 'Continuing treatment' listing where the most recent PBS authority approval for this PBS indication has been for gene therapy
Population criteria
Patient must have been 18 years of age or younger at the time of initial treatment with this drug;
AND
Population criteria
Patient must be 2 years of age or older and weigh 20 kg or more
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.
Prescribing Instructions: In a patient who wishes to switch from PBS-subsidised nusinersen to PBS-subsidised risdiplam for this condition a wash out period may be required.

Paediatric Pre-symptomatic SMA with 3 SMN2 gene copies

14639L; 14646W - Pre-symptomatic SMA (3 copies SMN2 gene) – Continuing/Maintenance

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	14639L HS	1	1	5	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	14646W HB	1	1	5	Evrysdi
risdiplam 5 mg tablet, 28	NEW (Private) NEW (Public)	1	28	5	Evrysdi
Restriction Summary [16044] / Treatment of Concept: [15986]					
Category / Program: ☒ Section 100 – Highly Specialised Drugs Program – Public (Code HB) / Private (Code HS)					
Prescriber type: ☒ Medical Practitioners					
Restriction type: ☒ Authority Required (immediate assessment) – telephone/online					
Authority type: ☒ Complex Authority Required (CAR)					
Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).					
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.					
Administrative Advice: No increase in the maximum number of repeats may be authorised.					
Administrative Advice: Special Pricing Arrangements apply.					
Indication: Pre-symptomatic spinal muscular atrophy (SMA)					
Treatment Phase Continuing/maintenance treatment of pre-symptomatic spinal muscular atrophy (SMA) with 3 copies of the SMN2 gene					
Clinical criteria:					

Patient must have previously received PBS-subsidised treatment with this drug for this condition; or
Patient must be eligible for continuing PBS-subsidised treatment with nusinersen for this condition
AND
Clinical criteria:
The treatment must not be in combination with PBS-subsidised treatment with nusinersen for this condition
AND
Clinical criteria:
The treatment must be given concomitantly with best supportive care for this condition
AND
Clinical criteria:
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
Treatment criteria:
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
AND
Treatment criteria:
Patient must not be undergoing treatment through this 'Continuing treatment' listing where the most recent PBS authority approval for this PBS indication has been for gene therapy
Population criteria
Patient must have been 18 years of age or younger at the time of initial treatment with this drug
AND
Population criteria
Patient must be 2 years of age or older and weigh 20 kg or more
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.
Prescribing Instructions: In a patient who wishes to switch from PBS-subsidised nusinersen to PBS-subsidised risdiplam for this condition a wash out period may be required.
Prescribing Instructions: The approved Product Information recommended dosing is as follows: 2 years of age and older weighing 20 kg or more: 5 mg

Paediatric Symptomatic type IIIB/IIIC SMA

13633M; 13639W – Paediatric Symptomatic type IIIB/IIIC SMA (Initial)

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13633M HB	3	3	5	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13639W HS	3	3	5	Evrysdi
risdiplam 5 mg tablet, 28	NEW (Private) NEW (Public)	1	28	5	Evrysdi
Restriction Summary [14395] / Treatment of Concept: [14408]					
Category / Program: ☒ Section100 – Highly Specialised Drugs Program – Public (Code HB) / Private (Code HS)					
Prescriber type: ☒ Medical Practitioners					
Restriction type: ☒ Authority Required (FULL assessment) in writing only via post/HPOS upload)					
Authority type: ☒ Complex Authority Required (CAR)					
Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday)., Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au, Applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos, Or mailed to:, Services Australia, Complex Drugs, Reply Paid 9826, HOBART TAS 7001					
Administrative Advice: An outcome on the authority application is not immediate, but will follow in due course. Electronic upload is encouraged to reduce processing time.					
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.					
Administrative Advice: No increase in the maximum number of repeats may be authorised.					
Administrative Advice: Special Pricing Arrangements apply.					
Indication: Symptomatic type IIIB/IIIC spinal muscular atrophy (SMA)					
Treatment Phase Initial PBS-subsidised treatment with this drug in a child					
Population criteria:					
Patient must be of an age that is prior to their 19th birthday at the time of this authority application;					
AND					
Population criteria:					
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);					
AND					
Population criteria:					
Patient must be 2 years of age or older and weigh 20 kg or more					
Clinical criteria:					
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					
Clinical criteria:					
Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug					

Treatment criteria:
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
AND
Treatment criteria:
Patient must be undergoing initial PBS-subsidised treatment with this drug for untreated disease; or
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)
AND
Treatment criteria:
Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.
Prescribing Instructions: The authority application must be made in writing and 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).
Prescribing Instructions: Signs and symptoms of spinal muscular atrophy in the context of this PBS restriction are:, (i) Failure to meet or regression in ability to perform age-appropriate motor milestones, (ii) Proximal weakness, (iii) Hypotonia, (iv) Absence of deep tendon reflexes, (v) Any active denervation or chronic neurogenic changes found on electromyography, (vi) A compound muscle action potential below normative values for an age-matched child.
Prescribing Instructions: In this authority application, confirm:, (1) the patient's medical history is consistent with a diagnosis of type IIIB/IIIC spinal muscular atrophy, (2) which of the above (i to vi) (at least 1) were present after their 3rd birthday, but before their 19th birthday, (3) the age of the patient (rounded to the nearest year) when the first sign/symptom was observed.

13631K 13659X - Paediatric/Adult Symptomatic type IIIB/IIIC SMA (Continuing/maintenance)

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
RISDIPLAM					
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13631K HB	3	3	5	Evrysdi
risdiplam 750 microgram/mL powder for oral liquid, 80 mL	13659X HS	3	3	5	Evrysdi
risdiplam 5 mg tablet, 28	NEW (Private) NEW (Public)	1	28	5	Evrysdi
Restriction Summary [14394] / Treatment of Concept: [14392]					
Category / Program: ☒ S100 – Highly Specialised Drugs Program – Public (Code HB) / Private (Code HS)					
Prescriber type: ☒ Medical Practitioners					
Restriction type: ☒ Authority Required (immediate assessment) – telephone/online					
Authority type: ☒ Complex Authority Required (CAR)					
Administrative Advice: Applications for authorisation under this restriction may be made in real time using the Online PBS Authorities system (see www.servicesaustralia.gov.au/HPOS) or by telephone by contacting Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).					
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.					
Administrative Advice: No increase in the maximum number of repeats may be authorised.					
Administrative Advice: Special Pricing Arrangements apply.					
Indication: Symptomatic type IIIB/IIIC spinal muscular atrophy (SMA)					
Treatment Phase Continuing/maintenance treatment in a child or adult, but where treatment was initiated during childhood					
Clinical criteria:					
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					
Treatment criteria:					
Patient must be undergoing continuation of existing PBS-subsidised treatment with this drug; or					
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					
Treatment criteria:					
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					
AND					
Treatment criteria:					
Patient must be undergoing concomitant treatment with best supportive care, but this benefit is the sole PBS-subsidised disease modifying treatment					
Population criteria:					
Patient must be 2 years of age or older and weigh 20 kg or more					
Prescribing Instructions: Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day.					

These restrictions may be subject to further review. Should there be any changes made to the restriction the sponsor will be informed.

8 Context for Decision

The PBAC helps decide whether and, if so, how medicines should be subsidised through the Pharmaceutical Benefits Scheme (PBS) in Australia. It considers applications regarding the listing of medicines on the PBS and provides advice about other matters relating to the operation of the PBS in this context. A PBAC decision in relation to PBS listings does not necessarily represent a final PBAC view about the merits of the medicine or the circumstances in which it should be made available through the PBS. The PBAC welcomes applications containing new information at any time.

9 Sponsor's Comment

The sponsor had no comment.