PB 70 of 2022

National Health (Highly Specialised Drugs Program) Special Arrangement Amendment (August Update) Instrument 2022

National Health Act 1953

I, NIKOLAI TSYGANOV, Assistant Secretary (Acting), Pricing and PBS Policy Branch, Technology Assessment and Access Division, Department of Health and Aged Care, delegate of the Minister for Health and Aged Care, make this Instrument under subsection 100(2) of the *National Health Act 1953*.

Date 28 July 2022

NIKOLAI TSYGANOV
Assistant Secretary (Acting)
Pricing and PBS Policy Branch
Technology Assessment and Access Division

Contents

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1 Name

(1) This instrument is the *National Health (Highly Specialised Drugs Program) Special Arrangement Amendment (August Update) Instrument 2022*.

(2) This instrument may also be cited as PB 70 of 2022.

2 Commencement

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

Commencement information		
Column 1	**Column 2**	**Column 3**
Provisions	**Commencement**	**Date/Details**
1. *The whole of this instrument*	*1 August 2022*	*1 August 2022*

Note: This table relates only to the provisions of this instrument as originally made.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

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

3 Authority

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

4 Schedules

Each instrument that is specified in a Schedule to this instrument is amended or repealed as set out in the applicable items in the Schedule concerned, and any other item in a Schedule to this instrument has effect according to its terms.

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Schedule 1—Amendments

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

[1] Schedule 1, entry for Nusinersen

(a) *omit from the column headed "Circumstances":* **C12643**

(b) *insert in numerical order in the column headed "Circumstances":* **C13046 C13047 C13064 C13089**

[2] Schedule 1, entry for Risdiplam

(a) *omit from the column headed "Circumstances":* **C12682**

(b) *insert in numerical order in the column headed "Circumstances":* **C13048**

[3] Schedule 2, entry for Nusinersen

substitute:

Nusinersen	C13047 C13064 C13089	1 dose	0
	C12667 C12672 C12676 C13046	1 dose	3

[4] Schedule 2, entry for Risdiplam *[Maximum quantity: 1; Maximum repeats: 5]*

omit from the column headed "Circumstances": **C12682** *substitute:* **C13048**

[5] Schedule 3, entry for Nusinersen

(a) *omit:*

	C12643		Spinal muscular atrophy (SMA) Continuing/maintenance treatment of either symptomatic Type I, II or IIIa SMA, or of a patient commenced on this drug under the pre-symptomatic SMA listing Must be treated by a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic of a recognised hospital in the management of SMA; or in consultation with a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic of a recognised hospital in the management of SMA; or initiated by a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic of a recognised hospital in the management of SMA; AND 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. 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 risdiplam for this condition; AND	Compliance with Written Authority Required procedures

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			The treatment must not be in combination with PBS-subsidised treatment with risdiplam for this condition; AND The treatment must be given concomitantly with best supportive care for this condition; AND 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. Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day. In a patient who wishes to switch from PBS-subsidised risdiplam to PBS-subsidised nusinersen for this condition a wash out period may be required.	

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

	C13046		Spinal muscular atrophy (SMA) Initial treatment of symptomatic SMA - Loading doses Must be treated by a specialist medical practitioner experienced in the diagnosis and management of SMA; or in consultation with a specialist medical practitioner experienced in the diagnosis and management of SMA. The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR The condition must have genetic confirmation of deletion of one copy of the SMN1 gene in addition to a pathogenic/likely pathogenic variant in the remaining single copy of the SMN1 gene; AND Patient must have experienced at least two of the defined signs and symptoms of SMA prior to 19 years of age; AND The treatment must not be used in combination with other SMA disease-modifying treatments, including risdiplam, for this condition; AND Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug; AND The treatment must be given concomitantly with best supportive care for this condition; AND The treatment must not exceed four loading doses (at days 0, 14, 28 and 63) under this restriction. Patient must be 19 years of age or older. Defined signs and symptoms of SMA are: (i) Onset before 19 years of age; and (ii) Failure to meet or regression in ability to perform age-appropriate motor milestones; or (iii) Proximal weakness; or (iv) Hypotonia; or (v) Absence of deep tendon reflexes; or (vi) Failure to gain weight appropriate for age; or (vii) Any active chronic neurogenic changes; or (viii) A compound muscle action potential below normative values for an age-matched child. Application for authorisation of initial treatment must be in writing and must include: (a) a completed authority prescription form; and (b) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice) which includes the following: (i) specification of SMA type; 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).	Compliance with Written Authority Required procedures

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	C13047		Spinal muscular atrophy (SMA) Continuing/maintenance treatment of symptomatic spinal muscular atrophy (SMA) Must be treated by a specialist medical practitioner experienced in the diagnosis and management of SMA; or in consultation with a specialist medical practitioner experienced in the diagnosis and management of SMA. Patient must have previously initiated PBS-subsidised treatment with this drug for this condition at the age of 19 years or older; AND The treatment must not be used in combination with other SMA disease-modifying treatments, including risdiplam, for this condition; AND The treatment must be given concomitantly with best supportive care for this condition; AND The treatment must be ceased when invasive permanent assisted ventilation is required in the absence of a potentially reversible cause while being treated with this drug; AND Patient must demonstrate a clinically meaningful response to treatment, following 2 years of treatment. Prior to continuing treatment, a comprehensive assessment must be undertaken and documented, involving the patient and the treating physician to establish agreement that treatment is continuing to produce worthwhile benefit. Treatment should cease if there is no agreement of benefit. Re-assessments for a clinically meaningful response are to be undertaken and documented every six months. Clinically meaningful response to treatment is defined as improvement, stabilisation or minimal decline in symptoms as demonstrated in the following areas: Maintenance of motor function as assessed using Revised Upper Limb Module (RULM), Hammersmith Functional Motor Scale - Expanded (HFMSE) and/or 6-minute walk test (6MWT). Maintenance of patient's quality of life including but not limited to level of independence. This may be informed by completion of the patient reported outcome measures SMA health index (SMA-HI) or SMA functional rating scale (SMAFRS).	Compliance with Authority Required procedures
	C13064		Spinal muscular atrophy (SMA) Continuing/maintenance treatment of either symptomatic Type I, II or IIIa SMA, or of a patient commenced on this drug under the pre-symptomatic SMA listing Must be treated by a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic of a recognised hospital in the management of SMA; or in consultation with a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic of a recognised hospital in the management of SMA; or initiated by a specialist medical practitioner experienced in the diagnosis and management of SMA associated with a neuromuscular clinic of a recognised hospital in the management of SMA; AND 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. 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 risdiplam for this condition; AND The treatment must not be in combination with PBS-subsidised treatment with risdiplam for this condition; AND The treatment must be given concomitantly with best supportive care for this condition; AND 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. Patient must have been 18 years of age or younger at the time of initial treatment with this drug. Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16	Compliance with Written Authority Required procedures

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			hours per day. In a patient who wishes to switch from PBS-subsidised risdiplam to PBS-subsidised nusinersen for this condition a wash out period may be required.	
	C13089		Spinal muscular atrophy (SMA) Transitioning from non-PBS to PBS-subsided treatment - Grandfather treatment Patient must have received non-PBS-subsidised treatment with this drug for this PBS indication prior to 1 August 2022. Must be treated by a specialist medical practitioner experienced in the diagnosis and management of SMA; or in consultation with a specialist medical practitioner experienced in the diagnosis and management of SMA. The condition must have genetic confirmation of 5q homozygous deletion of the survival motor neuron 1 (SMN1) gene; OR The condition must have genetic confirmation of deletion of one copy of the SMN1 gene in addition to a pathogenic/likely pathogenic variant in the remaining single copy of the SMN1 gene; AND Patient must have experienced at least two of the defined signs and symptoms of SMA prior to 19 years of age; AND The treatment must not be used in combination with other SMA disease-modifying treatments, including risdiplam, for this condition; AND Patient must not be receiving invasive permanent assisted ventilation in the absence of a potentially reversible cause while being treated with this drug; AND The treatment must be given concomitantly with best supportive care for this condition. Patient must be 19 years of age or older at the time of initial treatment with this drug. Defined signs and symptoms of SMA are: (i) Onset before 19 years of age; and (ii) Failure to meet or regression in ability to perform age-appropriate motor milestones; or (iii) Proximal weakness; or (iv) Hypotonia; or (v) Absence of deep tendon reflexes; or (vi) Failure to gain weight appropriate for age; or (vii) Any active chronic neurogenic changes; or (viii) A compound muscle action potential below normative values for an age-matched child. Application for authorisation of initial treatment must be in writing and must include: (a) a completed authority prescription form; and (b) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice) which includes the following: (i) specification of SMA type; 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). A patient may qualify for PBS-subsidised treatment under this restriction once only. For continuing PBS-subsidised treatment, a Grandfathered patient must qualify under the Continuing treatment criteria.	Compliance with Written Authority Required procedures

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[6] Schedule 3, entry for Risdiplam

(a) *omit:*

	C12682		Symptomatic Type I, II or IIIa spinal muscular atrophy (SMA) Continuing treatment 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 The treatment must not be in combination with PBS-subsidised treatment with nusinersen for this condition; AND The treatment must be ceased when invasive permanent assisted ventilation is required in the absence of a potentially reversible cause while being treated with this drug; AND The treatment must be given concomitantly with best supportive care for this condition. 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 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. Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day. 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.	Compliance with Written Authority Required procedures

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

	C13048		Symptomatic Type I, II or IIIa spinal muscular atrophy (SMA) Continuing treatment 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 The treatment must not be in combination with PBS-subsidised treatment with nusinersen for this condition; AND The treatment must be ceased when invasive permanent assisted ventilation is required in the absence of a potentially reversible cause while being treated with this drug; AND The treatment must be given concomitantly with best supportive care for this condition. 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 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. Patient must have been 18 years of age or younger at the time of initial treatment with this drug. Invasive permanent assisted ventilation means ventilation via tracheostomy tube for greater than or equal to 16 hours per day. 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.	Compliance with Written Authority Required procedures

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